



# Guide to Expanded Access



# About This Guide



For patients who are seriously ill and have exhausted available treatment options, and are not eligible for clinical trials, **Expanded Access** may be a potential option to explore with their physician. In response to patient feedback, Janssen created the Guide to Expanded Access (Guide) to support patients, caregivers and health care providers in Canada and European countries seeking information about **Expanded Access**. In collaboration with patient representatives, this Guide was created to support anyone in search of country-specific information about **Expanded Access** (EA), also known as **Compassionate Use**, or **Pre-Approval Access** (PAA).



This first of its kind Guide includes country-specific information for 28 European countries as well as Canada and includes information including but not limited to whether or not **Expanded Access** is available as a potential option and the process for requests in each country.

## How to Use This Guide

Information for each country may be accessed from the Table of Contents (**page 4**) by clicking on links to each section.



A glossary of key terms is also included (**pages 101-109**). If you are unfamiliar with **Expanded Access**, you may find it helpful to review the glossary first. To go back and forth between the Table of Contents and the Glossary, click the icons at the bottom of each page.



## Additional Resources about Expanded Access

For more information about **Expanded Access** at Janssen as well as other tools and resources, please visit <https://www.janssen.com/compassionate-use-pre-approval-access>



If you are seeking information about **Expanded Access** in the United States, please visit <https://www.fda.gov/news-events/public-health-focus/expanded-access>



# About This Guide (cont.)

## Question, Comment?

Patient and caregiver feedback will continue to inform the content in this Guide. We sincerely hope you will share your thoughts and experience with the EA Guide, and encourage you to send us an email at [PAASupport@its.jnj.com](mailto:PAASupport@its.jnj.com) to share your thoughts.

## Acknowledgement & Appreciation

Janssen would like to thank our colleagues at **Bionical Emas** for their strong collaboration and shared patient focus in bringing this Guide to patients, caregivers and health care providers.

## Disclaimers

The information in this Guide is intended solely for educational and informational purposes. It is not intended as medical or healthcare advice, or to be used for medical diagnosis or treatment for any individual problem. It is also not intended as a substitute for professional advice and services from a qualified healthcare provider familiar with your unique facts. Always seek the advice of your doctor or other qualified healthcare provider regarding any medical condition and before starting any new treatment.

We assume no responsibility for any consequence relating directly or indirectly to any action or inaction you take based on the information, or other material provided as part of the Services. While we strive to keep the information provided by the Services to be accurate, complete, and up to date, we do not give any assurances, and will not be responsible for, any damage or loss related to the accuracy, completeness, or timeliness of the information provided as part of the Services.

# Table of Contents

## Denmark

Page 27

## UK

Page 98

## Ireland

Page 55

## Belgium

Page 8

## France

Page 39

## Portugal

Page 80

## Spain

Page 91

## Malta

Page 72

## Germany

Page 44

## Austria

Page 5

## Italy

Page 58

## Croatia

Page 19

## Greece

Page 47

## Bulgaria

Page 12

## Cyprus

Page 20

## Luxembourg

Page 68

## Netherlands

Page 75

## Estonia

Page 31

## Finland

Page 35

## Sweden

Page 94

## Latvia

Page 62

## Lithuania

Page 65

## Poland

Page 79

## The Czech Republic

Page 24

## Slovakia

Page 87

## Slovenia

Page 88

## Hungary

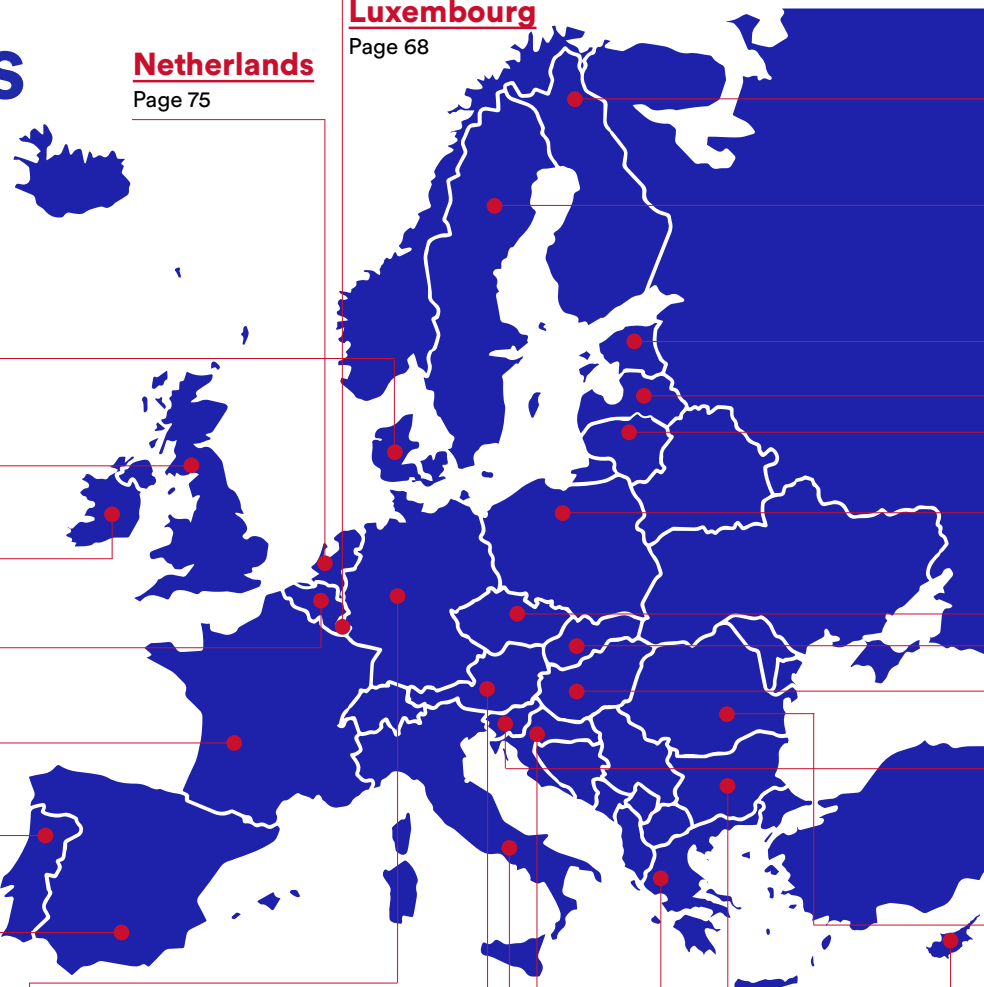
Page 51

## Romania

Page 84

## Canada

Page 15





**1. Is compassionate use a potential option in Austria?**

Yes, it is possible for a medicine that is not approved for use in Austria to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Austria?**

For the compassionate use of a medicine in Austria, it is required that:

- a. The medicine is not approved for use in Austria
- b. The manufacturer has applied to the **health authority** for approval of the medicine, or the medicine is being studied in **clinical trials**, and the manufacturer commits to apply for approval in the near future
- c. The pharmaceutical company sets up a compassionate use program that is approved in advance by **BASG** (the **health authority** in Austria)
- d. The patient must have a life-threatening illness or a seriously/chronically debilitating disease
- e. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** to enroll in the compassionate use program

In some urgent situations, it is possible for a patient to receive an unapproved medicine without a compassionate use program being set up in advance (see point c above); the patient's **treating physician** would need to explain why the medicine is urgently needed, and the pharmaceutical company must still agree to supply the medicine.



### 3. What is the usual process for requesting compassionate use of a medicine in Austria?

The following steps are required to request compassionate use of a medicine when a compassionate use program is in place:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the medicine can be shipped to the medical facility ready for patient treatment

Conversely, if an approved compassionate use program is not in place, the patient's **treating physician** provides a prescription and justification for use to the pharmacist to enable shipment of the product.

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to BASG or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

The time required for a patient to receive a medicine for compassionate use varies but can range from a few days to weeks. This is dependent on many factors, including but not limited to, the urgency of the treatment need, and importation requirements.



**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Austria and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine in a compassionate use program can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Austria for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Austria, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Belgium?**

Yes, it is possible for a medicine that is not approved for use in Belgium to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Belgium?**

For the compassionate use of a medicine in Belgium the following requirements must be met:

- a. The medicine is not approved for use in Belgium
- b. The medicine is being studied in **clinical trials**, or an application for approval of the medicine has been submitted to the **health authority** by the manufacturer
- c. The patient must have a life-threatening illness or a serious/chronic debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company has set up a ‘compassionate use program’ that has been approved by the **Federal Agency for Medicines and Health Products (FAMHP)** - the **health authority** in Belgium
- f. The patient meets established **eligibility criteria** for the compassionate use
- g. The patient’s **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- h. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient’s consent
- i. The Responsible Physician (a physician appointed by the pharmaceutical company for the compassionate use program) approves the enrolment of the patient into the program





In urgent and exceptional circumstances, when a compassionate use program is not possible or is not yet approved, it may still be possible for a patient to be treated with an unapproved medicine in individual cases. Agreement from the pharmaceutical company to supply the medicine is required, and the **treating physician** must request the medicine from the company.

### 3. What is the usual process for requesting compassionate use of a medicine in Belgium?

The following steps are required to request use of an unapproved medicine in Belgium, when an approved compassionate use program is in place:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician submits a request for enrolment into the program to the pharmaceutical company
- c. The request is medically reviewed and approved by the Responsible Physician who has been appointed by the pharmaceutical company
- d. The medicine is shipped to the hospital pharmacy

When an unapproved medicine is being requested for an urgent situation (described in question 2), i.e., not within an approved compassionate use program, the physician must obtain informed consent from the patient and submit required documentation to the supplier to enable shipment of the medicine.

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.



**4. Can a patient submit a request to FAMHP or a pharmaceutical company?**

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

**5. How long does the process take?**

If a request is approved, the time taken for a patient to be treated with an unapproved medicine varies and is dependent on many factors. Factors including, but not limited to, importation requirements and Responsible Physician approval (see questions 2 and 3), may contribute to the total time before a patient is able to be treated.

**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is "**commercially available**" i.e., approved for use, in Belgium and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Belgium for one disease, it is possible to receive the medicine on a compassionate use basis for another disease if the following requirements are met:

- a. **Clinical trials** are underway, an application has been submitted to the **health authority** for the use of the medicine for the disease, or the medicine has been approved but is not yet **commercially available**
- b. The patient must have a life-threatening disease, a chronic disease or a disease with a serious impact
- c. The patient cannot be treated with available medicines
- d. The pharmaceutical company has set up a 'medical need program' that has been approved by **FAMHP** (the **health authority** in Belgium)
- e. The patient meets established **eligibility criteria** for the program
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The Responsible Physician (a physician appointed by the pharmaceutical company for the medical need program) approves the enrolment of the patient into the program

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Belgium, the patient usually must be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Bulgaria?**

Yes, it is possible for a medicine that is not approved for use in Bulgaria to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Bulgaria?**

For the compassionate use of a medicine in Bulgaria, it is required that:

- a. The medicine is not approved for use in Bulgaria
- b. The medicine is being studied in **phase 3 clinical trials** (exceptionally, **phase 2 clinical trials**), or the manufacturer of the medicine has submitted an application to the **health authority** for approval of the medicine
- c. A compassionate use program, approved in advance by the **Bulgarian Drugs Agency** (the **health authority** in Bulgaria), has been set up by the pharmaceutical company
- d. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- e. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use





### 3. What is the usual process for requesting compassionate use of a medicine in Bulgaria?

The following steps are required to request compassionate use of a medicine in Bulgaria when a compassionate use program has been set up (see question 2):

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the pharmaceutical company confirms the patient is eligible, the medicine can be shipped to the medical facility

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to the Bulgarian Drugs Agency or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If a compassionate use program is in place, the time required for a patient to receive the medicine can vary and is dependent on several factors including, but not limited to, importation requirements.

**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Bulgaria and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Bulgaria for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Bulgaria, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Canada?**

Yes, it is possible for a medicine that is not approved for use in Canada to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Canada?**

For the compassionate use of a medicine in Canada, it is required that:

- a. The medicine is not approved for use in Canada
- b. The patient must have a life-threatening or serious disease
- c. The patient cannot be treated with medicines that are approved for use in Canada or that are available through a **clinical trial**
- d. The pharmaceutical company commits to supply the medicine in a written agreement with **Health Canada** (the **health authority** in Canada)
- e. The patient's **treating physician** considers that the use of the medicine is sufficiently supported by clinical data, its use in the patient is justified and the benefits outweigh the risks
- f. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- g. The patient meets established **eligibility criteria** for the compassionate use
- h. **Health Canada** approves the use of the medicine for an individual patient, following a request from the **treating physician**



### 3. What is the usual process for requesting compassionate use of a medicine in Canada?

The following steps are required to request compassionate use of a medicine in Canada:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the pharmaceutical company and reviews the available clinical data supporting the use of the medicine before deciding to prescribe
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician prepares a request to **Health Canada**, the **health authority** in Canada
- d. **Health Canada** reviews the request, and if approved, the medicine can be shipped to the medical facility

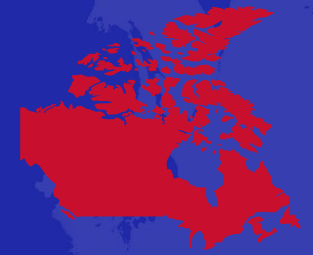
There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to Health Canada or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.





### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply and has an agreement in place with **Health Canada** (the **health authority** in Canada), a physician request may be reviewed by **Health Canada** as quickly as within 24 hours. Following **Health Canada** approval, the medicine will then need to be shipped to the medical facility. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

### 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Canada and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

### 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Canada for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Canada, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.

Patient Guide  
**CROATIA**



**1. Is compassionate use a potential option in Croatia?**

There is currently no legislation that allows for the compassionate use of an unapproved medicine in Croatia. Access to unauthorised medicines is limited to **clinical trials** only. Patients are encouraged to discuss treatment options with their **treating physician**.



**1. Is compassionate use a potential option in Cyprus?**

Yes, it is possible for a medicine that is not approved for use in Cyprus to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Cyprus?**

For the compassionate use of a medicine in Cyprus, it is required that:

- a. The medicine is not approved for use in Cyprus
- b. The pharmaceutical company agrees to provide the medicine
- c. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- d. The patient meets established **eligibility criteria** for the compassionate use
- e. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- f. The **health authority** in Cyprus, the **Ministry of Health**, approves a request submitted by the **treating physician** for the use of the medicine for the individual patient





### 3. What is the usual process for requesting compassionate use of a medicine in Cyprus?

The following steps are required to request compassionate use of a medicine in Cyprus:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician prepares a request to the **Ministry of Health** (the **health authority** in Cyprus)
- d. The **Ministry of Health** reviews the request, and if approved, the medicine can be shipped to the hospital

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to the Ministry of Health or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. There is no standard review timeline set by the **Ministry of Health** (the **health authority** in Cyprus) once a request has been submitted by the **treating physician**; each request is assessed on a case by case basis and prioritised accordingly. Following **Ministry of Health** approval, the medicine will then need to be shipped to the hospital for patient treatment. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

### 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Cyprus and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

### 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Cyprus for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

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**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Cyprus, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



## 1. Is compassionate use a potential option in the Czech Republic?

Yes, it is possible for a medicine that is not approved for use in the Czech Republic to be made available for compassionate use, so long as certain requirements are met (see question 2).

## 2. What are the key requirements for compassionate use in the Czech Republic?

For the compassionate use of a medicine in the Czech Republic, it is required that:

- a. The medicine is not approved for use in the Czech Republic
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application to the **health authority** to request approval of the medicine
- c. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company sets up a 'specific therapeutic program' (STP) that has been approved by the **Ministry of Health** and the State Institute of Drug Control (SUKL) (health authorities in the Czech Republic)
- f. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- g. The physician considers that the use of the medicine is justified, and the benefits outweigh the risks
- h. The patient meets established **eligibility criteria** for enrolment into the STP



### 3. What is the usual process for requesting compassionate use of a medicine in the Czech Republic?

The following steps are required to request compassionate use of a medicine in the Czech Republic:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the pharmaceutical company providing the medicine
- c. If the company confirms the patient is eligible, the medicine can be shipped to the medical facility

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to SUKL or a pharmaceutical company?

No. A request for compassionate use must be submitted to the pharmaceutical company and only by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

The time required for a patient to receive the medicine within a specific therapeutic program varies and is dependent on factors including, but not limited to, importation requirements.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in the Czech Republic and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in the Czech Republic for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of the Czech Republic, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.





**1. Is compassionate use a potential option in Denmark?**

Yes, it is possible for a medicine that is not approved for use in Denmark to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Denmark?**

For the compassionate use of a medicine in Denmark, it is required that:

- a. The medicine is not approved for use in Denmark
- b. The patient cannot be treated with approved and available medicines
- c. The pharmaceutical company agrees to provide the medicine
- d. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- e. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- f. The patient meets established **eligibility criteria** for the compassionate use
- g. The **health authority** in Denmark, the **Danish Medicines Agency (DMA)**, issues a permit to allow the use of the medicine, following an application from the patient's **treating physician**



### 3. What is the usual process for requesting compassionate use of a medicine in Denmark?

The following steps are required to request compassionate use of a medicine in Denmark:

- a. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The patient's **treating physician** makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician prepares a request to the **Danish Medicines Agency (DMA)** providing justification for use
- d. The **DMA** reviews the request, and if approved, the medicine is shipped to the medical facility and can be dispensed by the pharmacy for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to the Danish Medicines Agency or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply, and for which a request has been made to the **Danish Medicines Agency (DMA)**, the approval time can be 3-4 weeks. However, for urgent cases, **DMA** approval can be much quicker. Following **DMA** approval, the medicine will then need to be shipped to the medical facility for patient treatment. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

### 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Denmark and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

### 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Denmark for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

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**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Denmark, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Estonia?**

Yes, it is possible for a medicine that is not approved for use in Estonia to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Estonia?**

For the compassionate use of a medicine in Estonia, it is required that:

- a. The medicine is not approved for use in Estonia (or is approved for use, but in a different disease)
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application to the **health authority** for approval of the medicine
- c. The medicine is being used as a last treatment option for a life-threatening illness or a chronic or disabling disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a clinical trial
- e. The pharmaceutical company agrees to provide the medicine and notifies the **State Agency of Medicines (SAM)**, the **health authority** in Estonia, of the compassionate use program in advance
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use
- i. The **SAM** has approved a request for use of the medicine, submitted by the patient's **treating physician**



### 3. What is the usual process for requesting compassionate use of a medicine in Estonia?

Once a pharmaceutical company has notified the program to the **State Agency of Medicines (SAM)**, the **health authority** in Estonia, the following steps are required to request compassionate use of a medicine in Estonia:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician submits a compassionate use request to the pharmaceutical company
- c. If the company confirms the patient is eligible, the physician submits a prescription and justification for use of the medicine to the **SAM**
- d. The **SAM** reviews the application and notifies the physician of their decision
- e. If the application is approved, an import application must be submitted to the **SAM** by the distributor of the medicine before it can be imported into Estonia and delivered to the medical facility for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

### 4. Can a patient submit a request to the SAM or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.





### 5. How long does the process take?

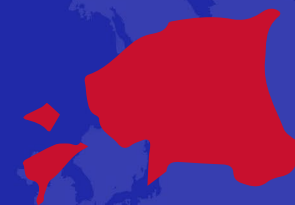
The time required for a patient to be treated with an unapproved medicine in Estonia varies, and is dependent on many factors. If a pharmaceutical company has notified the compassionate use program to the **State Agency of Medicines (SAM)**, the **health authority** in Estonia, then a physician's application is typically reviewed within 14-30 days. Following approval of the physician application by the **SAM** the medicine must be delivered to the medical facility; if this involves importation of the medicine from another country then import approval from the **SAM** usually takes a further 5 days.

### 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Estonia and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

### 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Estonia for one disease, it may be possible for a patient to be treated with the medicine on a compassionate basis for a different disease. The conditions outlined in question 2 must still be met.

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**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Estonia, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.

**1. Is compassionate use a potential option in Finland?**

Yes, it is possible for a medicine that is not approved for use in Finland to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Finland?**

For the compassionate use of a medicine in Finland, it is required that:

- a. The medicine is not approved for use in Finland
- b. The patient cannot be treated with approved and available medicines or available medicines do not achieve the desired effect
- c. The pharmaceutical company agrees to provide the medicine
- d. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- e. The patient meets established **eligibility criteria** for the compassionate use
- f. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- g. The **treating physician** provides medical justification for use of the medicine
- h. The **health authority** in Finland, **FIMEA**, provides a permit to release the medicine for use of the medicine for the individual patient



### 3. What is the usual process for requesting compassionate use of a medicine in Finland?

The following steps are required to request compassionate use of a medicine in Finland:

- a. The patient's **treating physician** makes a compassionate use request to the company providing the medicine
- b. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician prescribes the medicine and provides the prescription and justification to the pharmacy
- d. The pharmacy applies to **FIMEA** (the **health authority** in Finland) for permission to release the product to the patient
- e. **FIMEA** reviews the request, and, if approved, the medicine can be shipped to the medical facility for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

### 4. Can a patient submit a request to FIMEA or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



## 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply, and for which an application for a permit has been made to **FIMEA** (the **health authority** in Finland), the request is typically processed within 30 days. However, for urgent cases, **FIMEA** approval may be much quicker. Following **FIMEA** approval the medicine will then need to be shipped to the medical facility. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Finland and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Finland for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

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**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Finland, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.





**1. Is compassionate use a potential option in France?**

Yes, it is possible for a medicine that is not approved for use in France to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in France?**

For the compassionate use of a medicine in France, it is required that:

- a. The medicine is not approved for use in France, or is approved for use in a different disease
- b. The manufacturer has submitted an application for approval of the medicine to the **health authority**, or commits to submit an application within a set timeframe
- c. There are supporting data that strongly indicate the medicine will be effective and safe in the treatment of the disease
- d. The patient must have a serious or rare disease with no suitable available treatment
- e. The treatment of the patient cannot be postponed
- f. The pharmaceutical company agrees to provide the medicine
- g. An application for a ‘Temporary Authorization for Use’ (ATU) is submitted by the pharmaceutical company for a cohort of patients and approved in advance by **ANSM** (the **health authority** in France)
- h. A protocol, which defines how the patients will be treated and monitored, is agreed between **ANSM** and the pharmaceutical company as part of the application process
- i. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient’s consent
- j. The patient’s **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- k. The patient meets established **eligibility criteria** for the ATU



In some exceptional circumstances, it may be possible for a patient to be treated with an unapproved medicine based on an individual request submitted to **ANSM** by the **treating physician**; this is known as a nominative ATU. This may be possible when the patient cannot enter a clinical trial, or when a cohort ATU is not in place, and the medicine is expected to be of benefit to the patient by the **treating physician**. There must be supporting data that indicate the medicine will be effective and safe in the treatment of the disease. Usually, the nominative ATU can only be issued if a request for a cohort ATU has already been submitted to **ANSM**, OR if an application for the general use of the medicine in France has been submitted to the **health authority**, OR if a clinical trial is ongoing in France.

### 3. What is the usual process for requesting compassionate use of a medicine in France?

The following steps are required to request compassionate use of a medicine in France when a cohort Temporary Authorisation for Use (see question 2) is in place:

- a. The patient's **treating physician** obtains the protocol, which defines how patients will be treated and monitored, from the pharmaceutical company or **ANSM** (the **health authority** in France)
- b. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- c. The pharmacist at the medical facility submits the treatment request to the pharmaceutical company
- d. The pharmaceutical company validates the request and supplies the medicine to the medical facility ready for patient treatment

If an individual patient is being treated under a nominative ATU (see question 2) the **treating physician** must submit a request directly to **ANSM**, which must be approved prior to treatment of the patient.

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.



#### 4. Can a patient submit a request to ANSM or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

#### 5. How long does the process take?

The time required for a patient to be treated with a medicine for compassionate use in France varies and is dependent on many factors.

For a medicine that has a cohort Temporary Authorisation for Use in place (see question 2), approval by **ANSM** (the **health authority** in France) is not required for each patient, and the medicine can be delivered to the medical facility for patient treatment as soon as the pharmaceutical company has validated the request.

For the treatment of an individual patient, when a cohort Temporary Authorisation for Use is not in place, the **treating physician's** request must be reviewed and approved by **ANSM**. The timeline for review by **ANSM** will depend on the urgency of the request (based on the information provided by the physician in the application). It will also depend upon whether the medicine has already been reviewed by **ANSM**. The review process can be as quick as 24-48 hours for very urgent cases. Once the request is approved, the medicine can then be delivered to the medical facility for patient treatment.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. In some instances, medicines may not be provided free of charge by the company, but the cost will be reimbursed by **ANSM** (the **health authority** in France), meaning that the patient will not need to pay for the medicine. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in France and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in France for one disease, it may be possible for a patient to be treated with the medicine on a compassionate basis for a different disease if a group Temporary Authorisation for Use is in place (see question 2). The conditions described in question 2 must still be met.



**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of France, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Germany?**

Yes, it is possible for a medicine that is not approved for use in Germany to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Germany?**

For the compassionate use of a medicine in Germany, it is required that:

- a. The medicine is not approved for use in Germany
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application to the **health authority** for approval of the medicine
- c. The patient must have a life-threatening illness or seriously debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- f. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- g. The pharmaceutical company agrees to provide the medicine and has set up a compassionate use program for a group of patients that has been approved by the relevant **health authority** in Germany
- h. The patient meets established **eligibility criteria** for the compassionate use





### 3. What is the usual process for requesting compassionate use of a medicine in Germany?

The following steps are required to request compassionate use of a medicine in Germany, when an approved compassionate use program is in place (see question 2):

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the medicine can be shipped to the medical facility for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to the health authority in Germany or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If a request is approved by the pharmaceutical company, the time required for a patient to receive the medicine varies. There is no **health authority** approval required for treatment of each patient; the time taken mainly depends on how long it takes for shipment of the medicine to the medical facility.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Germany and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Germany for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Germany, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.

**1. Is compassionate use a potential option in Greece?**

Yes, it is possible for a medicine that is not approved for use in Greece to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Greece?**

For the compassionate use of a medicine in Greece, it is required that:

- a. The patient must have a life-threatening or a chronic disabling disease
- b. The pharmaceutical company has set up an ‘early access program’ approved in advance by the National Organization of Medicines – the **health authority** in Greece
- c. The patient meets the established **eligibility criteria** to enter the early access program
- d. The medicine is not approved for use in Greece, or the medicine is approved but for a different condition or disease
- e. The manufacturer has submitted an application to the **health authority** for approval of the medicine OR **clinical trials** are underway, and the manufacturer commits to apply for approval of the medicine
- f. The patient cannot be treated with approved and available medicines
- g. All existing treatments have been exhausted
- h. The patient’s **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient’s consent
- i. The physician considers that the use of the medicine is justified, and the benefits outweigh the risks
- j. The physician has approval from the hospital to use the unauthorised medicine for the patient

If the patient is suffering from certain rare diseases or in extremely urgent cases, it is possible for the patient to receive an unapproved medicine without an early access program being set up in advance by the pharmaceutical company, (see point b above). In this case, the Greek **health authority** must approve an individual request from the patient's **treating physician**. The pharmaceutical company must still agree to supply the medicine for the individual patient, and the requirements d to j above, still apply.

### 3. What is the usual process for requesting compassionate use of a medicine in Greece?

The following steps are required to request compassionate use of a medicine when an early access program is in place:

- a. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician, with agreement from the hospital, makes a compassionate use request to the pharmaceutical company providing the medicine
- c. If the company confirms the patient is eligible, the medicine can be shipped to the clinic

If an early access program is not in place (see question 2), then, in addition to the steps outlined above, the physician must submit an individual application to the National Organisation of Medicines – the **health authority** in Greece - to obtain a permit.

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.

#### 4. Can a patient submit a request to the health authority or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

#### 5. How long does the process take?

The time required for a patient to receive a medicine for compassionate use varies and is dependent on many factors including, but not limited to, importation requirements and **health authority** approval (in cases where there is not an early access program in place).

#### 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is "**commercially available**" i.e., approved for use, in Greece and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

#### 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

It is possible to receive a medicine for compassionate use that is approved in Greece for a different disease, so long as the requirements outlined in question 2 are met.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Greece, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Hungary?**

Yes, it is possible for a medicine that is not approved for use in Hungary to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Hungary?**

For the compassionate use of a medicine in Hungary, it is required that:

- a. The medicine is not approved for use in Hungary
- b. The medicine is being studied in, or has completed, at least **phase 2 clinical trials** for the disease
- c. The patient must have a life-threatening or a chronic debilitating disease
- d. It is not possible for the patient to be treated with medicines that are approved and available in Hungary
- e. The patient cannot be treated within an ongoing **clinical trial** for the medicine
- f. The **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent prior to treating
- h. The pharmaceutical company has given consent for use in the patient and agrees to supply the medicine free of charge
- i. The pharmaceutical company guarantees the quality of the medicine
- j. A request for use of the medicine, submitted by the **treating physician**, is approved by **OGYÉI** (the **health authority** in Hungary)





### 3. What is the usual process for requesting compassionate use of a medicine in Hungary?

The following steps are required to request compassionate use of a medicine in Hungary:

- a. The patient's **treating physician** makes a compassionate use request to the pharmaceutical company providing the medicine
- b. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- c. The company confirms the patient is eligible and agrees to supply the medicine
- d. The physician prepares and submits a request to **OGYÉI** (the **health authority** in Hungary)
- e. **OGYÉI** reviews the request and notifies the physician and the medical facility's pharmacy of the decision
- f. If the request is approved, the medicine can be shipped to the medical facility for patient treatment

There may also be additional steps based on internal processes for the use of unapproved medicines at the medical facility.

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### 4. Can a patient submit a request to the health authority or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



## 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply, and for which a complete request has been submitted to **OGYÉI** (the **health authority** in Hungary) the approval time is up to 21 days. However, for situations that are considered by the **treating physician** to be medically urgent, approval can be as quick as 3 days. Following **OGYÉI** approval, the medicine will then need to be shipped to the medical facility. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Hungary and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Hungary for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Hungary, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Ireland?**

Yes, it is possible for a medicine that is not approved for use in Ireland to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Ireland?**

For the compassionate use of a medicine in Ireland, it is required that:

- a. The medicine is not approved for use in Ireland
- b. The medicine is being studied in **phase 3 clinical trials**, or the manufacturer has submitted an application to the **health authority** for approval of the medicine
- c. There is no approved equivalent medicine in Ireland
- d. The pharmaceutical company agrees to provide the medicine
- e. The medicine is only supplied in response to a request from the patient's **treating physician**
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use



### 3. What is the usual process for requesting compassionate use of a medicine in Ireland?

The following steps are required to request compassionate use of a medicine in Ireland:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician, makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine the medicine can be shipped to the hospital

There may also be additional steps based on internal processes for the use of unapproved medicines at the hospital.

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### 4. Can a patient submit a request to a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies, and is dependent on factors including, but not limited to, importation requirements.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Ireland and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Ireland for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Ireland, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Italy?**

Yes, it is possible for a medicine that is not approved for use in Italy to be made available for compassionate use so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Italy?**

For the compassionate use of a medicine in Italy, it is required that:

- a. The medicine is not approved for use in Italy (or the medicine is approved for use in Italy, but for a *different* condition or disease)
- b. The medicine is being studied in **clinical trials**. Usually the medicine is in the later stages of clinical development known as **phase 3 trials**, but in some life-threatening situations, and for rare diseases or rare cancer, it is possible for the medicine to be at an earlier stage of development
- c. The pharmaceutical company agrees to provide the medicine
- d. The patient must have a life-threatening or a seriously debilitating disease
- e. It is not possible for the patient to be treated with medicines that are approved and available in Italy
- f. The patient cannot be treated within an ongoing **clinical trial** for the medicine
- g. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- h. The patient meets established **eligibility criteria** for compassionate use of the medicine
- i. The local **Ethics Committee** approves the request
- j. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent





**3. What is the usual process for requesting compassionate use of a medicine in Italy?**

The following steps are required to request compassionate use of a medicine in Italy:

- a. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The patient's **treating physician** makes a compassionate use request to the company providing the medicine
- c. The company confirms that the patient is eligible and agrees to supply the medicine
- d. The physician prepares and submits a request to the local **Ethics Committee**
- e. The **Ethics Committee** reviews the request and notifies the physician of approval
- f. The physician provides the pharmaceutical company with the **Ethics Committee** approval, plus other required documentation, to allow the medicine to be shipped to the medical facility for patient treatment

There may also be additional steps based on internal processes for the use of unapproved medicines at the medical facility.

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**4. Can a patient submit a request to the Ethics Committee or a pharmaceutical company?**

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply, and for which a request has been submitted to the local **Ethics Committee**, the approval time varies by committee and can take a few weeks or even months, although urgent cases may be reviewed more quickly. Following **Ethics Committee** approval, the medicine will then need to be shipped to the medical facility. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

### 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Italy and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

### 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients will normally be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

It is possible to receive a medicine for compassionate use that is approved in Italy for a different disease or condition. This is dependent upon the pharmaceutical company agreeing to provide the medicine for that disease and the conditions described in question 2 being met.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Italy, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another a country will not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Latvia?**

Yes, it is possible for a medicine that is not approved for use in Latvia to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Latvia?**

For the compassionate use of a medicine in Latvia, it is required that:

- a. The medicine is not approved for use in Latvia
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. A request for distribution is submitted by the pharmaceutical company and approved by the **State Agency of Medicines** (the **health authority** in Latvia). The request must include a 'program of use', which contains all the relevant information about the medicine for the **treating physician**
- f. The **State Agency of Medicines** issues a permit to the healthcare institution for use of the medicine
- g. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- h. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- i. The patient meets established **eligibility criteria** for the compassionate use



### 3. What is the usual process for requesting compassionate use of a medicine in Latvia?

The following steps are required to request compassionate use of a medicine in Latvia when approval to distribute the medicine has been granted to the pharmaceutical company (see question 2):

- a. The patient's **treating physician** requests the 'program of use' from the pharmaceutical company (see question 2)
- b. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- c. The physician makes a compassionate use request to the company providing the medicine, providing a justification for the use of the medicine
- d. The company submits an application to the **State Agency of Medicines** (the **health authority** in Latvia) to add the medical facility to the compassionate use program
- e. The medicine is shipped to the medical facility for patient treatment

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### 4. Can a patient submit a request to the State Agency of Medicines or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

The time required for a patient to receive a medicine for compassionate use varies and is dependent on many factors including, but not limited to, the time taken for the **State Agency of Medicines** (the **health authority** in Latvia) to issue a permit to the medical facility and importation requirements.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Latvia and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Latvia for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Latvia, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Lithuania?**

Yes, it is possible for a medicine that is not approved for use in Lithuania to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Lithuania?**

For the compassionate use of a medicine in Lithuania, it is required that:

- a. The medicine is not approved for use in Lithuania
- b. The medicine is being studied in **clinical trials**
- c. The medicine is being used to save or prolong the life of a patient
- d. The pharmaceutical company agrees to provide the medicine
- e. The use of the medicine for the individual patient is approved by the hospital **Ethics Committee** and at least two disease specialists
- f. The use of the medicine for the individual patient is approved by the **State Medicines Control Agency of Lithuania** (the **health authority**)
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use





### 3. What is the usual process for requesting compassionate use of a medicine in Lithuania?

The following steps are required to request compassionate use of a medicine in Lithuania:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to provide the medicine, the physician submits an individual request to the **State Medicines Control Agency of Lithuania** (the **health authority**), following approval from the hospital **Ethics Committee** and two disease specialists
- d. If the request is approved, the medicine is shipped to the hospital for patient treatment

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.

### 4. Can a patient submit a request to the State Medicines Control Agency of Lithuania or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

### 5. How long does the process take?

The time required for a patient to receive a medicine for compassionate use varies and is dependent on many factors including, but not limited to, the time taken for the **State Medicines Control Agency of Lithuania** to approve the use of the medicine and importation requirements.

**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Lithuania and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Lithuania for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Lithuania, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



## 1. Is compassionate use a potential option in Luxembourg?

Yes, it is possible for a medicine that is not approved for use in Luxembourg to be made available for compassionate use, so long as certain requirements are met (see question 2).

## 2. What are the key requirements for compassionate use in Luxembourg?

For the compassionate use of a medicine in Luxembourg, it is required that:

- a. The medicine is not approved for use in Luxembourg
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company agrees to provide the medicine
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use
- i. A request for the use of the medicine for the individual patient is approved by the **Ministère-Direction de la Santé** (the **health authority** in Luxembourg)



### 3. What is the usual process for requesting compassionate use of a medicine in Luxembourg?

The following steps are required to request compassionate use of a medicine in Luxembourg:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician prepares a request to the **Ministère-Direction de la Santé** (the **health authority** in Luxembourg)
- d. The **Ministère-Direction de la Santé** reviews the request, and if approved, the medicine can be shipped to the hospital for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to the Ministère-Direction de la Santé or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



## 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply, and for which a request for use has been made to the **Ministère-Direction de la Santé**, the approval can be as quick as one day. Following approval from the **Ministère-Direction de la Santé**, the medicine will then need to be shipped to the hospital. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Luxembourg and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).



**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Luxembourg for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

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**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Luxembourg, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.

**1. Is compassionate use a potential option in Malta?**

Yes, it is possible for a medicine that is not approved for use in Malta to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Malta?**

For the compassionate use of a medicine in Malta, it is required that:

- a. The medicine is not approved for use in Malta
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The pharmaceutical company has set up a compassionate use program, approved in advance by the **Malta Medicines Authority** (the **health authority** in Malta)
- d. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- e. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use
- i. The **Malta Medicines Authority** approves the use of the medicine for the patient in advance, if required by the compassionate use program



### 3. What is the usual process for requesting compassionate use of a medicine in Malta?

The following steps are required to request compassionate use of a medicine in Malta when a compassionate use program is in place:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician may need to prepare a request to the **Malta Medicines Authority** (the **health authority** in Malta), depending on the requirements of the compassionate use program
- d. If the request is approved, the medicine can be shipped to the medical facility for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to the Malta Medicines Authority or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors including, but not limited to, approval time of requests by the **Malta Medicines Authority** and importation requirements.

## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Malta and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Malta for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Malta, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



## 1. Is compassionate use a potential option in the Netherlands?

Yes, it is possible for a medicine that is not approved for use in the Netherlands to be made available for compassionate use, so long as certain requirements are met (see question 2).

## 2. What are the key requirements for compassionate use in the Netherlands?

For the compassionate use of a medicine in the Netherlands, it is required that:

- a. The medicine is not approved for use in the Netherlands
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The patient must have a life-threatening illness or a progressive and severe disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company sets up a compassionate use program that is approved in advance by the **health authority** in the Netherlands
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use

If a compassionate use program has not been set up by the pharmaceutical company, a patient in the Netherlands may be able to receive a medicine for compassionate use if the company agrees to provide the medicine and the patient's **treating physician** provides a written declaration that justifies its need.



The doctor's declaration forms part of a request that the pharmaceutical company submits to the **health authority** in the Netherlands for approval. In these cases, there must be no alternative approved treatment options in the Netherlands.

### 3. What is the usual process for requesting compassionate use of a medicine in the Netherlands?

The following steps are required to request compassionate use of a medicine in the Netherlands when a compassionate use program is in place:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the medicine can be shipped to the medical facility for patient treatment

If a compassionate use program is not in place, in addition to the above, the physician must submit a written declaration for the justification of the use of the medicine for the individual patient to the pharmaceutical company. The pharmaceutical company then includes the doctor's declaration in a request for approval by the **health authority** on a per patient basis.

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.



**4. Can a patient submit a request to the health authority or a pharmaceutical company?**

No. A request for compassionate use must be submitted by the patient’s **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

**5. How long does the process take?**

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors including, but not limited to, importation requirements. For a medicine used within a compassionate use program the time taken is mainly due to the time required to import the medicine. If a compassionate use program is not in place, and the medicine is being requested under a doctor’s declaration (see question 2) then it can take up to 8 weeks for **health authority** approval. However, this may be quicker for urgent cases and the pharmaceutical company can request permission for a group of patients in advance so that approval per patient is not required.

**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in the Netherlands and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.



**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in the Netherlands for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of the Netherlands, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Poland?**

In exceptional circumstances, the **Ministry of Health** (the **health authority** in Poland) may approve the use of an unlicensed medicine that is currently undergoing **clinical trials**. In justified cases, individual patients may be treated with an unlicensed medicine when the patient's life or health is at risk and if the pharmaceutical company agrees to provide the medicine. Patients are encouraged to discuss treatment options with their **treating physician**.





**1. Is compassionate use a potential option in Portugal?**

Yes, it is possible for a medicine that is not approved for use in Portugal to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Portugal?**

For the compassionate use of a medicine in Portugal, it is required that:

- a. The medicine is not approved for use in Portugal OR is approved for use but not yet **commercially available** OR the medicine is approved for use but for a different disease
- b. If the medicine is not approved for use in Portugal, the medicine has proof of efficacy and safety from **clinical trials** in the disease that will be used to support an application to the **health authority** for approval of the medicine
- c. The patient must have a rare or serious or extremely debilitating disease
- d. The patient cannot be treated with approved and available medicines
- e. The pharmaceutical company sets up an early access program that has been approved in advance by **Infarmed** (the **health authority** in Portugal)
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use
- i. **Infarmed** approves a request, submitted by the hospital, for treatment of the patient with the medicine



If an early access program is not set up by the pharmaceutical company, a request can still be submitted to **Informed** by the hospital for the treatment of the patient with the unapproved medicine. In such cases the pharmaceutical company must agree to supply the medicine, the medicine must be essential for the patient's treatment and there must be no approved alternative.

### 3. What is the usual process for requesting compassionate use of a medicine in Portugal?

The following steps are required to request compassionate use of a medicine in Portugal:

- a. The hospital obtains approval from their Pharmacy and Therapeutics Committee for use of the medicine
- b. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- c. The physician makes a compassionate use request to the company providing the medicine
- d. If the company confirms the patient is eligible and agrees to supply the medicine, the clinical director of the hospital submits a request to **Informed** (the **health authority** in Portugal)
- e. If the request is approved, the medicine can be shipped to the hospital for patient treatment

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.

### 4. Can a patient submit a request to **Informed** or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.



## 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors, including but not limited to, **Infarmed** approval, delivering the medicine to the hospital and importation requirements.

## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Portugal and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Portugal for one disease it may be possible to obtain the medicine for compassionate use for another disease, so long as the conditions outlined in question 2 are met. Patients are encouraged to discuss treatment options with their **treating physician**.



**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Portugal, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Romania?**

Yes, it is possible for a medicine that is not approved for use in Romania to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Romania?**

For the compassionate use of a medicine in Romania, it is required that:

- a. The medicine is not approved for use in Romania
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company sets up a compassionate use program that is approved by the **National Agency for Medicines and Medical Devices of Romania (NAMMD)**, the **health authority** in Romania
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use
- i. The **NAMMD** approves the use of the medicine for the individual patient



### 3. What is the usual process for requesting compassionate use of a medicine in Romania?

The following steps are required to request compassionate use of a medicine in Romania when a compassionate use program is in place:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible, the pharmaceutical company submits a request to the **National Agency for Medicines and Medical Devices of Romania (NAMMD)**, the **health authority** in Romania
- d. **NAMMD** reviews the request and, if approved, the medicine can be shipped to the hospital for patient treatment

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to NAMMD or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors including, but not limited to, the time required for **NAMMD** approval and importation requirements.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Romania and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Romania for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Romania, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



Patient Guide  
**SLOVAKIA**



**1. Is compassionate use a potential option in Slovakia?**

There is currently no legislation that allows for the compassionate use of an unapproved medicine in Slovakia. Access to unapproved medicines is limited to **clinical trials** only. Patients are encouraged to discuss treatment options with their **treating physician**.



**1. Is compassionate use a potential option in Slovenia?**

Yes, very exceptionally, it is possible for a medicine that is not approved for use in Slovenia to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Slovenia?**

For the compassionate use of a medicine in Slovenia, it is required that:

- a. The medicine is not approved for use in Slovenia
- b. The patient must have a life-threatening illness or a severe or chronically debilitating disease that requires emergency treatment
- c. The patient cannot be treated with approved and available medicines
- d. The pharmaceutical company agrees to supply the medicine
- e. **JAZMP** (the **health authority** in Slovenia) approves the import of the medicine for the treatment of an individual patient under the direct responsibility of the patient's **treating physician**
- f. The physician considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use



### 3. What is the usual process for requesting compassionate use of a medicine in Slovenia?

The following steps are required to request compassionate use of a medicine in Slovenia:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible, a request is submitted to **JAZMP** (the **health authority** in Slovenia) with a statement from the physician explaining the justification for use of the medicine
- d. If the request is approved, the medicine can be shipped to the medical facility ready for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to **JAZMP** or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. If the pharmaceutical company has agreed to supply the medicine and a request has been submitted to **JAZMP** (the **health authority** in Slovenia) the approval can take up to 30 days. Following **JAZMP** approval the medicine will then need to be shipped to the medical facility. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.



**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Slovenia and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Slovenia for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Slovenia, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Spain?**

Yes, it is possible for a medicine that is not approved for use in Spain to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Spain?**

For the compassionate use of a medicine in Spain, it is required that:

- a. The medicine is not approved for use in Spain
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company agrees to provide the medicine
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use
- i. The **health authority** in Spain, **Agencia Española de Medicamentos y Productos Sanitarios (AEMPS)**, approves a request for use of the medicine for the individual patient, submitted by the hospital



**3. What is the usual process for requesting compassionate use of a medicine in Spain?**

The following steps are required to request compassionate use of a medicine in Spain:

- a. The physician ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The patient's **treating physician**, with agreement from the hospital, makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the physician prepares a request to **AEMPS** (the **health authority** in Spain), which is then submitted by the hospital pharmacy via an electronic portal
- d. **AEMPS** reviews the request, and if approved, the medicine can be shipped to the hospital

There may be additional steps required at the hospital due to local procedures on the use of unapproved medicines.

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**4. Can a patient submit a request to AEMPS or a pharmaceutical company?**

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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**5. How long does the process take?**

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors. For a medicine that a pharmaceutical company has agreed to supply, and for which a complete submission has been made to **AEMPS** (the **health authority** in Spain), the approval typically takes a maximum of 7 days. However, for urgent cases, **AEMPS** approval may be quicker. Following **AEMPS** approval the medicine will then need to be shipped to the hospital. Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.



**6. Will I need to pay for the compassionate use medicine?**

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Spain and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

**7. How long can a patient receive a medicine on a compassionate basis?**

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

**8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?**

Once a medicine has been approved for use in Spain for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. However, there may be exceptional situations that require assessment by **AEMPS** (the **health authority** in Spain) on a case-by-case basis. Patients are encouraged to discuss treatment options with their **treating physician**.

**9. Can a patient apply for compassionate use in a country where they do not reside?**

To obtain a medicine via compassionate use in a country outside of Spain, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



**1. Is compassionate use a potential option in Sweden?**

Yes, it is possible for a medicine that is not approved for use in Sweden to be made available for compassionate use, so long as certain requirements are met (see question 2).

**2. What are the key requirements for compassionate use in Sweden?**

For the compassionate use of a medicine in Sweden, it is required that:

- a. The medicine is not approved for use in Sweden
- b. The medicine is being studied in **clinical trials**, or the manufacturer has submitted an application for approval of the medicine to the **health authority**
- c. The patient must have a life-threatening illness or a severe or chronically debilitating disease
- d. The patient cannot be treated with approved and available medicines, and is ineligible to participate in a **clinical trial**
- e. The pharmaceutical company has set up a compassionate use program that has been approved in advance by the **Medical Product Agency (MPA)** – the **health authority** in Sweden
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's consent
- h. The patient meets established **eligibility criteria** for the compassionate use

If a compassionate use program has not been set up by a pharmaceutical company, then approval of the use of medicine by the **MPA** is required on a per patient basis. An application must be submitted to the **MPA** by the **treating physician** which must demonstrate that the patient cannot be treated with approved medicines or they are ineffective.

### 3. What is the usual process for requesting compassionate use of a medicine in Sweden?

The following steps are required to request compassionate use of a medicine in Sweden when a compassionate use program is in place:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment and obtains the patient's written consent
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, it can be shipped to the medical facility for patient treatment

If a compassionate use program is not in place (see question 2) in addition to the steps outlined above, a justification for use of the product must be prepared by the patient's **treating physician** and a request submitted to, and approved by, the **MPA** (the **health authority** in Sweden).

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

### 4. Can a patient submit a request to the MPA or a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

## 5. How long does the process take?

If a request is approved, the time required for a patient to receive the medicine varies and is dependent on many factors.

For a medicine used within an approved compassionate use program (see question 2) there is no approval required per patient by the **MPA** (the **health authority** in Sweden). Therefore, as soon as the company confirms the patient is eligible, the medicine can be shipped to the medical facility.

Where there is no compassionate use program in place, an approval by the **MPA** is required on an individual basis. This can take 7 working days, or longer in some instances, if it is the first request for the product the **MPA** has reviewed. In urgent cases, the approval time is reduced and can be approved the same day.

Other factors including, but not limited to, importation requirements may contribute to the total time before a patient is able to be treated.

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## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in Sweden and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.



## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in Sweden for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of Sweden, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



## 1. Is compassionate use a potential option in the UK?

Yes, it is possible for a medicine that is not approved for use in the UK to be made available for compassionate use, so long as certain requirements are met (see question 2).

## 2. What are the key requirements for compassionate use in the UK?

For the compassionate use of a medicine in the UK, it is required that:

- a. The medicine is not approved for use in the UK
- b. The patient has a special need that can be met by the medicine
- c. There is no equivalent approved and available medicine in the UK that can meet the special need of the patient
- d. The pharmaceutical company agrees to provide the medicine
- e. The **MHRA** (the **health authority** in the UK) has been notified in advance that the medicine will be imported into the UK
- f. The patient's **treating physician** considers that the use of the medicine is justified, and the benefits outweigh the risks
- g. The **treating physician** ensures the patient understands the benefits and risks of treatment
- h. The patient meets established **eligibility criteria** for the compassionate use

Additionally, for promising medicines intended to treat seriously debilitating or life-threatening conditions where there are no adequate treatment options, pharmaceutical companies can set up a program under the **Early Access to Medicines Scheme (EAMS)**. This is only an option for patients where an **EAMS**, that has been evaluated and approved by the **MHRA**, is in place.



### 3. What is the usual process for requesting compassionate use of a medicine in the UK?

The following steps are required to request compassionate use of a medicine in the UK:

- a. The patient's **treating physician** ensures the patient understands the benefits and risks of treatment to enable an informed decision
- b. The physician makes a compassionate use request to the company providing the medicine
- c. If the company confirms the patient is eligible and agrees to supply the medicine, the medicine can be shipped to the medical facility for patient treatment

There may be additional steps required at the medical facility due to local procedures on the use of unapproved medicines.

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### 4. Can a patient submit a request to a pharmaceutical company?

No. A request for compassionate use must be submitted by the patient's **treating physician**. Patients are encouraged to speak with their **treating physician** if they feel compassionate use may be an option.

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### 5. How long does the process take?

If an **Early Access to Medicines Scheme (EAMS)** is in place, and the pharmaceutical company has confirmed the patient is eligible, the patient's **treating physician** can prescribe the medicine in the usual way. If an **EAMS** is not in place, the time required for a patient to receive the medicine varies and is dependent on factors including, but not limited to, importation requirements.



## 6. Will I need to pay for the compassionate use medicine?

In most circumstances, medicines for compassionate use are provided free of charge by the pharmaceutical company. Compassionate use is generally provided only up until the medicine is “**commercially available**” i.e., approved for use, in the UK and available to be prescribed by a physician. After the medicine is **commercially available**, patients are responsible for payment of the medicine through their customary process for prescription medicines.

## 7. How long can a patient receive a medicine on a compassionate basis?

A patient receiving a medicine on a compassionate use basis can usually do so until the medicine becomes **commercially available**, so long as the benefits of treatment continue to outweigh the risks, and the patient can tolerate the treatment. When the medicine becomes **commercially available**, compassionate use may end. From this time, patients may be supplied the medicine through customary methods for prescription medicines, and charges may apply. It is important to note that supply via compassionate use may need to be discontinued for other reasons (e.g., safety concerns).

## 8. What if a patient has a need for compassionate use of a medicine that is approved for a different disease?

Once a medicine has been approved for use in the UK for one disease, the medicine cannot usually be obtained via compassionate use for a different disease. Patients are encouraged to discuss treatment options with their **treating physician**.

## 9. Can a patient apply for compassionate use in a country where they do not reside?

To obtain a medicine via compassionate use in a country outside of the UK, the patient must usually be a resident in that country. A local **treating physician** must also agree to submit a request to the local **health authority** (depending on regulatory requirements in that country). Moving to another country does not guarantee access to a medicine, even if the medicine is available via compassionate use in that country.



# Patient Guide

# GLOSSARY

## **Agencia Española de Medicamentos y Productos Sanitarios (AEMPS)**

The AEMPS is a state agency in Spain attached to the Ministry of Health. It is responsible for guaranteeing to society, from a public service perspective, the quality, safety, efficacy and correct information of medicines and health products, from their research to their use, in the interests of protecting and promoting human health, animal health and the environment. For more information: <https://www.aemps.gob.es/la-aemps/quienes-somos/?lang=en>

## **ANSM**

The National Agency for the Safety of Medicines and Health Products is the public actor which, on behalf of the State, allows access to health products in France and which ensures their safety throughout their life cycle. For more information: [https://www.ansm.sante.fr/L-ANSM/Une-agence-d-expertise/L-ANSM-agence-d-evaluation-d-expertise-et-de-decision/\(offset\)/0](https://www.ansm.sante.fr/L-ANSM/Une-agence-d-expertise/L-ANSM-agence-d-evaluation-d-expertise-et-de-decision/(offset)/0)

## **BASG**

BASG is the national authority for drugs, medical devices, blood and tissue in Austria. The BASG monitors - nationally and in concert with the European sister agencies - the drugs and medical devices that are already on the market with regard to their effectiveness, possible side effects, their production, transport and storage. For more information: <https://www.basg.gv.at/ueber-uns>

## **Bulgarian Drugs Agency**

The Bulgarian Drug Agency at the Ministry of Healthcare is defined as a body for the supervision of the quality, efficiency and safety of medicines. For more information: <https://www.bda.bg/en/about-bda/history#from-nimp-to-bda>

## **Clinical Trial(s)**

Interventional study (clinical trial) is a study in which participants are assigned to groups that receive one or more intervention/treatment (or no intervention) so that researchers can evaluate the effects of the interventions on biomedical or health-related outcomes. The assignments are determined by the study's protocol. Participants may receive diagnostic, therapeutic, or other types of interventions. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

# GLOSSARY (cont.)

## ClinicalTrials.gov

ClinicalTrials.gov is an online database of publicly and privately supported clinical trials conducted around the world. Doctors and patients may consult this database when seeking access to an experimental drug, via either a clinical trial or pre-approval access. While ClinicalTrials.gov is operated by the United States National Institutes of Health (NIH), being listed in the database should not be seen as an endorsement by the NIH or the U.S. Food and Drug Administration (FDA) of the value of any agent or product.

## Commercially Available

A product that has received local health authority approval and the product is able to be prescribed.

## Compassionate Use

“Compassionate use” is access to investigational drugs that have not yet been approved for sale or use by the U.S. Food and Drug Administration (FDA) or relevant regulatory authority, such as Health Canada. The term is often used interchangeably with “expanded access” and “pre-approval access.”

Often investigational drugs may be available to patients through clinical trials. Compassionate use is the provision of the drug to patients who are unable to participate in a clinical trial because of severity of illness or some other factor.

Sponsors decide whether to make their investigational drugs available to patients via compassionate use. Should a sponsor, often a private company, be willing to provide the investigational drug, the FDA must approve the planned use of that drug. FDA regulations specify two groups of people eligible for compassionate use: 1) those with life-threatening diseases or conditions for which “there is a reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment” and 2) those with serious diseases or conditions that have a “substantial impact on day-to-day functioning” (21 Code of Federal Regulations 312.300(b)). In most cases, patients who seek compassionate use must have exhausted all approved therapies for their condition and be unable to enroll in a clinical trial.

# GLOSSARY (cont.)

## Danish Medicines Agency (DMA)

The Danish Medicines Agency authorises and inspects pharmaceutical companies and licenses medicinal products in the Danish market, monitors adverse reactions from medicinal products, authorises clinical trials, decides which medicines are eligible for reimbursement, monitors medical devices available in Denmark and supervises adverse incidents involving medical devices, appoints proprietary pharmacists, organises the pharmacy structure and supervises pharmacies and retailers. They perform most of their tasks in close cooperation with colleagues from regulatory authorities and organisations in the other EU countries. For more information: <https://laegemiddelstyrelsen.dk/en/about/>

## Early Access to Medicines Scheme (EAMS)

The Early Access to Medicines Scheme (EAMS) in the United Kingdom aims to make promising new medicines available to patients sooner. It was set up in 2014 and is run by the Medicines and Healthcare Products Regulatory Agency (MHRA). For more information: <https://www.cancerresearchuk.org/about-cancer/cancer-in-general/treatment/access-to-treatment/early-access-to-medicines-scheme>

## Eligibility Criteria

Eligibility criteria are the key requirements for entry into a clinical study or other investigational medicine program such as expanded access. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

## Ethics Committee

Ethics committees are a group of individuals formed to protect the interests of patients and address moral issues. It normally includes a board member of the institution, a lay person, and an administrator. Most ethics committees work in an advisory capacity; they can help patients and families reach informed decisions and work with health care providers in order to make complex and difficult decisions.

## Expanded Access

“Expanded access” (EA) is the U.S. Food and Drug Administration’s (FDA) term for access outside clinical trials to investigational drugs that the agency has not yet approved. Expanded access is an umbrella term that applies to single patient requests and programs for groups, either intermediate-size or larger. Treatment of the patient, rather than collection of data, is the primary goal. This is also known as a “Group” or “Cohort” program.

# Patient Guide

## GLOSSARY (cont.)

### Expanded Access Programs (EAPs)

EAPs are designed to permit larger groups of patients to access an investigational drug. For both single patient requests and EAPs, treatment of the patient, rather than collection of data, is the primary goal. However, in an EAP, data are frequently collected from patients enrolled in the program.

### Federal Agency for Medicines and Health Products (FAMHP)

The FAMHP is the competent authority responsible for the quality, safety and efficacy of medicines and health products in Belgium. For more information: <https://www.famhp.be/en/famhp>

### FIMEA

Operating under the Ministry of Social Affairs and Health, the Finnish Medicines Agency Fimea oversees and develops the pharmaceutical sector. For more information: [https://www.fimea.fi/web/en/for\\_public/fimea-s-services-to-citizens](https://www.fimea.fi/web/en/for_public/fimea-s-services-to-citizens)

### Form FDA 3926

Form 3926 is a U.S. Food and Drug Administration (FDA) form for use by physicians when submitting requests for expanded access to investigational drugs, including emergency requests. This form is designed specifically for single patient requests, not for EAPs. In 2016, this form was created as a shorter, streamlined alternative to Form FDA 1571, which still must be used for EAPs.

### Health Authority

A Health Authority is a government agency that is responsible for national health services care in a particular area.

### Health Canada

Health Canada is a federal institution that is part of the Health portfolio that is responsible for helping Canadians maintain and improve their health. It ensures that high-quality health services are accessible, and works to reduce health risks.

# GLOSSARY (cont.)

## Individual Request

Named patient requests based on an application made by the physician and/or local importer e.g., pharmacy or wholesaler.

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## Infarmed

National Authority for Medicines and Health Products, IP, abbreviated as Infarmed, is a public institute with a special regime, under the terms of the law, integrated in the indirect administration of the State, endowed with administrative, financial and own assets. Infarmed continues the duties of the Ministry of Health, under the supervision and supervision of the respective minister. For more information: <https://www.infarmed.pt/web/infarmed/apresentacao>

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## Institutional Review Board (IRB)

An IRB, sometimes referred to as a research ethics committee, is a committee charged with reviewing, approving, and monitoring biomedical and behavioral research involving humans. In the U.S., pre-approval access requires review and approval by an IRB. IRB review comes after the drug company agrees to provide access to an investigational product and the FDA reviews and accepts the proposed treatment plan.

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## Investigational New Drug (IND)

Federal law requires that a drug or other therapeutic agent be approved for use before it can be transported and distributed in the U.S. An IND exemption is the means through which a sponsor obtains permission from the FDA to distribute the agent before it has this approval. Under an IND, sponsors may distribute an investigational drug to study it in clinical trials needed for approval. An IND must also be submitted to receive access to an investigational drug for compassionate use.

There are four types of expanded access INDs: 1) individual patient expanded access, which allows compassionate use of a drug by a single patient; 2) Emergency Use IND, which allows the FDA to authorize the use of an investigational drug in an emergency situation, as it did during the 2014 Ebola outbreak; 3) Intermediate-Size Patient Population IND, which allows multiple patients to gain compassionate use access to an investigational drug, and 4) Treatment IND, which is submitted for the widespread use of investigational drugs showing promise in clinical testing for serious or immediately life-threatening conditions.

# GLOSSARY (cont.)

## JAZMP

JAZMP of Slovenia's primary mission is to protect public health through the regulation and supervision of medicinal products, medical devices, blood, tissues and cells and associated activities in the private and public sector. For more information: <https://www.jazmp.si/en/>

## Malta Medicines Authority

Malta's Medicines Authority's vision is to be a centre of excellence in advancing effective and innovative regulation and promoting quality and scientific rigour in the work we do. For more information: <http://www.medicinesauthority.gov.mt/missionobjectives?l=1>

## Medical Product Agency (MPA)

The Medical Products Agency of Sweden is the responsible for regulation and surveillance of the development, manufacturing and sale of drugs and other medicinal products. For more information: <https://www.government.se/government-agencies/medical-products-agency-lakemedelsverket-lv/>

## MHRA

The Medicines and Healthcare products Regulatory Agency regulates medicines, medical devices and blood components for transfusion in the UK.

## Ministère-Direction de la Santé

The missions of the Ministère-Direction de la Santé, the Ministry of Health of Luxembourg, are the definition and application of government health policy, monitoring the application of health laws and regulations, supervision of health institutions and services. For more information: <https://sante.public.lu/fr/politique-sante/ministere-sante/index.html>

## Ministry of Health

The Ministry of Health of Poland is a government administration office that supports the minister of health. For more information: <https://www.gov.pl/web/zdrowie/podstawowe-informacje>

## Named Patient Program

A program to provide access on a named patient (individual) or group/cohort basis.

# GLOSSARY (cont.)

## National Agency for Medicines and Medical Devices of Romania (NAMMD)

The National Agency for Medicines and Medical Devices (NAMMD) is a public institution subordinated to the Ministry of Health of Romania. The NAMMD mission is to help protect and promote public health. For more information: <https://www.anm.ro/en/despre-institutie/despre-noi/>

## OGYÉI

OGYÉI is responsible for the tasks performed by the General Directorate of the GYEMSZI Institute of Pharmacy, the Directorate of Device Qualification and Hospital Technology, the Technology Evaluation Department, the GYEMSZI, the ÁNTSZ and the National Food and Nutrition Institute for Food and Nutrition. For more information: <https://ogyei.gov.hu/magunkrol>

## Phase 2 Clinical Trial(s)

Phase 2 Clinical Trial is a phase of research to describe clinical trials that gather preliminary data on whether a drug works in people who have a certain condition/disease (that is, the drug's effectiveness). For example, participants receiving the drug may be compared to similar participants receiving a different treatment, usually an inactive substance (called a placebo) or a different drug. Safety continues to be evaluated, and short-term adverse events are studied. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

## Phase 3 Clinical Trial(s)

Phase 3 Clinical Trial is a phase of research to describe clinical trials that gather more information about a drug's safety and effectiveness by studying different populations and different dosages and by using the drug in combination with other drugs. These studies typically involve more participants. For more information: <https://www.clinicaltrials.gov/ct2/about-studies/glossary>

# GLOSSARY (cont.)

## Pre-Approval Access (PAA)

PAA is an umbrella term encompassing access to investigational medicines, such as expanded access programs and compassionate use. PAA refers to any use of unapproved drugs outside of clinical trials, particularly if the intent is therapeutic rather than to gain data (research).

## Single Patient Request (SPR)

Investigational access request outside of a clinical trial for one patient outside of an expanded access program.

## Sponsor

A sponsor is the person or entity that takes responsibility for and initiates a clinical trial of an investigational agent. In the context of pre-approval access, the sponsor is typically a pharmaceutical or biotech company.

## State Agency of Medicines (SAM) Estonia

State Agency of Medicines is a governmental body under the Ministry of Social Affairs in Estonia. Its main responsibility is the protection and promotion of public and animal health, through the supervision of medicines for human and veterinary use. For more information: <https://www.ravimiamet.ee/en/sam>

## State Agency of Medicines (SAM) Latvia

The State Agency of Medicines of Latvia (SAMLV) is a State institution under the supervision of the Ministry of Health of the Republic of Latvia. The operational objective of SAMLV is to implement local and international pharmaceutical legislation in order to ensure that the products (medicines, medical devices, blood, cells, tissues and organs) used in health care, as well as the involved companies and their activities comply with certain requirements. For more information: <https://www.zva.gov.lv/en/about-us/about-agency>



# GLOSSARY (cont.)

**State Medicines  
Control Agency  
of Lithuania**

The State Medicines Control Agency (SMCA) is a governmental body of the Republic of Lithuania with headquarters in Vilnius. Its main responsibility is the protection of public health, through the evaluation and supervision of medicines for human use. For more information:

<https://www.vvkt.lt/index.php?2380224066>

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**Treating Physician**

Treating Physician is the doctor who is providing medical treatment for a condition specific to expanded access requests.