# EARLY ACCESS POLICY

## *Clinical Trial Access*

At Orphazyme, we aim to positively impact the lives of patients and their families with rare diseases by developing innovative therapies. With our treatments in development, we strive to help as many patients as quickly as is possible.

Obtaining regulatory approval and marketing authorization by authorities such as the FDA in the United States and the EMA in the European Union is essential to our providing broad access to our treatments. For obtaining regulatory approval, clinical trials are required to evaluate the risks and benefits of the drug in development. Until the drug receives regulatory authority approval it remains investigational and cannot be commercially available for the treatment of patients.

Our goal is to complete a clinical trial program to demonstrate the safety and efficacy of the investigational drug, then to obtain the regulatory approvals needed for these drugs to become available to patients who need them.

We also understand that patients facing urgent medical need and caregivers are keen to swiftly obtain access to our product, even if still in the investigational phase. The preferred access to our investigational drug is by participation in one of our clinical trials. For those individuals able to so participate we are committed to providing continued access to the drug through the appropriate pathway until regulatory approval is achieved and the drug commercialized or reimbursed in their country, or until the development program is terminated for an unfavorable risk:benefit assessment.

For more information on our clinical trials, please visit: https://www.clinicaltrials.gov.

*Please note that our clinical trials in Niemann-Pick Type C (NPC), Amyotrophic Lateral Sclerosis (ALS) and Inclusion Body Myositis (IBM) have completed recruitment.*

## ORPHAZYME EARLY ACCESS PROGRAM CHARTER

We recognize that not all patients are able to participate in our clinical trials and do face urgent medical need. Depending on the regulations in the patient's country of residence and based on specific eligibility criteria (if appropriate to the program offered, see below), Orphazyme will consider offering organized 'Early Access' or 'Compassionate Use' Programs providing access to an investigational drug outside of a clinical trial before the drug has obtained approval and becomes commercially available.

We aim to ensure that such access is provided in an ethical and fair manner while minimizing risk both to the individual patient and to the broader patient community in the future.

**Named Patient Programs (NPPs):** for further information on individual named patient access to our investigational drug please contact us: medicalaffairs@orphazyme.com

### For these reasons Early Access Programs can only be offered:

- When substantial scientific evidence from clinical trials exists to support both the safety and the efficacy of a therapy for an indication and the potential benefits to the patient from the investigational therapy are greater than the potential risks.
- When it neither compromises the investigational therapy's development program nor the pathway for regulatory approval.
- Where Orphazyme intends to seek regulatory approval, an appropriate regulatory framework is established and a market access pathway for rare diseases exists and it is possible for an organised programme to be established.*
- When Orphazyme is able to maintain an adequate and sustainable supply of the investigational therapy, beyond the requirement for ongoing and planned clinical trial completion.

*N.B. Please note that this Charter also covers France where Early Access is provided by the nominative Temporary Use Authorization (nATU).*

## Early Access Program Patient Eligibility

***In order to gain Early Access to an Orphazyme investigational drug, the following criteria must be met:***

a. The patient has a serious or life-threatening medical condition within the disease area and patient population that Orphazyme is investigating.

b. There are no satisfactory alternative therapies available to the patient.

c. The patient is neither eligible to participate in ongoing or planned clinical trials in a reasonably accessible geographical location nor are there any suitable clinical trials currently open for recruitment.

d. The patient has a disease for which there is scientific rationale for a possible therapeutic benefit and that such benefit is likely to outweigh any known/anticipated risk.

e. The patient seeking Early Access is a full-time resident of the country in which an Early Access program is approved and operating; in some countries the patient needs to have access to publicly funded healthcare services in that country.

f. The patient seeking Early Access fulfills the EAP/CUP protocol *(*for protocol driven programs)* eligibility criteria, as confirmed by the treating physician.

g. The request for Early Access is received from a qualified and licensed physician with expertise in the disease and who understands the potential risks and benefits of the investigational drug.

h. The requesting physician agrees to comply with Orphazyme's Early Access specifications (as above) as well as applicable laws and regulations governing the use of the investigational drug.

*N.B. Early Access in any country ends once the investigational drug is commercially available and/or reimbursed in that country.*

Should Orphazyme discontinue clinical development of the investigational drug for a specific disease area, Early Access will be discontinued.

## Current Early Access to Arimoclomol

1. **Niemann-Pick Type C (NPC):** Early Access to arimoclomol is currently available in the USA, France and Germany.
2. **Other conditions for which arimoclomol is being studied:** (Amyotrophic Lateral Sclerosis, Inclusion Body Myositis and Gaucher disease): Orphazyme does not have any currently active Early Access to arimoclomol programs.

### Procedure to Request Early Access to Arimoclomol for Patients with NPC

Orphazyme has partnered with the Clinigen Group to coordinate arimoclomol Early Access. For further information on these programs, see below:

- **United States:** US Healthcare Professionals can obtain details about the arimoclomol EAP or make a request for early access for their patient by calling **Clinigen customer service team at +1 877-768-4303 or emailing usmapoperations@clinigengroup.com.**
- **European Union:** EU Healthcare Professionals can obtain details about existing Early Access to arimoclomol from publicly available information provided by the competent regulatory authority. To obtain additional information or make a request for Early Access for their patient, healthcare professionals should please contact:
- **Clinigen customer service team at**
  - **France: +33 (0) 1573 23223**
  - **Germany: +49 (0) 6922 223413or email managedaccess@clinigengroup.com**

Please note that the initial request should not contain personally identifiable information about the patient (e.g. name, contact details, personal identification number). Receipt of the request will be acknowledged within five business days.

Patients seeking medical information should either contact their physician or medicalaffairs@orphazyme.com.

**GDPR and Privacy Statement:**

The information Orphazyme receives as part of any email communication will be held under strict confidentiality and managed in accordance with the EU's General Data Protection Regulations (GDPR).

We encourage you to carefully read our Privacy Statement that describes how Orphazyme processes your personal data and explains your rights in relation to the personal data processed by Orphazyme. You may find the Privacy Statement at www.orphazyme.com.