

# Independent Medical Education Request for Education (RFE)

## Key information

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| <b>Date Issued:</b>               | August 5, 2025  |
| <b>RFE Requestor Information:</b> | Sonja Boon, CHCP<br>Amicus Therapeutics<br>Ph: 848-213-7406<br>Email: sboon@amicusrx.com  |
| <b>RFE Code:</b>                  | RFE-PD-25-110   |
| <b>Geographic Scope:</b>          | Global medical society meeting taking place in the United States. If distribution of enduring materials is expanded to audiences beyond US for the enduring activity, include a break-down of costs for the additional geographies.   |
| <b>RFE Response Due Date:</b>     | September 2, 2025   |
| <b>Amicus IME Portal:</b>         | <a href="https://www.cybergrants.com/amicus/IME">https://www.cybergrants.com/amicus/IME</a>   |
| <b>Area of Interest:</b>          | Late-Onset Pompe Disease (LOPD)   |
| <b>Educational Venue:</b>         | <ul style="list-style-type: none"><li>• Live satellite symposium at <i>WORLDSymposium 2026</i>, San Diego, CA</li><li>• Interactive live with enduring web-based components will be considered</li><li>• Accredited continuing medical education activities</li></ul>   |
| <b>Educational Audiences:</b>     | The education should address the needs of clinicians who have a role in the management of patients with LOPD including but not be limited to neuromuscular specialists, geneticists, neurologists, lysosomal disease specialists, genetic counselors, pediatricians, NPs, PAs and other healthcare professionals. |
| <b>Program Budget:</b>            | Single-supported proposals with a request amount that does not exceed \$370,000 will be considered. Live symposium, including symposium fee by WORLD and costs of the enduring activity should be included in the budget.   |
| <b>Accreditation:</b>             | ACCME, NSGC and/or others as appropriate  |

## Needs Assessment

Pompe disease is a rare, lysosomal disease caused by pathogenic mutations in the acid alpha-glucosidase (GAA) gene.<sup>1</sup> This leads to deficiency of the GAA enzyme and multisystemic accumulation of lysosomal glycogen, predominantly in skeletal muscle.<sup>1,2</sup> Late-onset Pompe disease (LOPD) is heterogenous, but most patients experience involvement of muscles in the proximal lower and upper limbs, paraspinal muscles, and diaphragm, and initial clinical manifestations often include difficulty walking and climbing stairs.<sup>1-3</sup> Disease progression is variable with the danger that irreversible muscle damage can lead to reduced movement and breathing ability<sup>3,4</sup> and patients may ultimately require ambulatory aids and/or ventilatory assistance.<sup>1-3</sup> LOPD places a heavy burden on patients and their families; patients frequently experience decreased health-related quality of life and physical health, as well as social and relational issues.<sup>5-8</sup>

A number of management and treatment guidelines exist for LOPD, including from US and EU-based consortia.<sup>9-14</sup> The guidelines recommend initiating treatment soon after symptom onset and the use of a multidisciplinary team for regular, holistic monitoring.<sup>9-14</sup> Many guidelines have not been updated following the approval of new treatments for LOPD;<sup>9-13,15-21</sup> updates are expected to include these advancements along with new monitoring recommendations within the evolving treatment landscape.

While there have been considerable advances in the field of LOPD including new monitoring techniques,<sup>22–32</sup> the development of new treatment options<sup>15–21</sup> and investigational therapies,<sup>33,34</sup> and an increasing focus on shared-decision making, several challenges remain.

These include, but are not limited to:

- Delays to diagnosis<sup>3,9,13,35</sup>
- Monitoring and management of patients with LOPD diagnosed via newborn screening<sup>36–38</sup>
- Improving monitoring of patients with LOPD, including those who are asymptomatic and/or those who may have subtle signs of progression despite being on treatment, eg using novel techniques such as MRI and ultrasound and video- or device-based techniques as potential ways to monitor motor and respiratory function<sup>22–32</sup>
- Understanding the utility of biomarkers (eg hexose tetrasaccharide [Hex4] and creatine kinase [CK]) in monitoring treatment response<sup>3,39,40</sup>
- Differentiating between the current treatment options, including the two more recent therapy options (where available) in the absence of a head-to-head trial<sup>14</sup>

Additional education could therefore be beneficial to HCPs who are managing individuals living with LOPD, or those who work in specialties where an improved understanding of the disease could potentially lead to more prompt diagnosis and effective patient management.

## Healthcare Gaps

***Note: It is expected that any education provider submitting a grant application conduct their own independent needs assessment when identifying gaps in patient care and learning objectives that aim to reduce those gaps.***

Insights from past program outcomes, a detailed literature search, and educational needs assessment have established the need to address remaining practice gaps to improve the quality of care for patients around the globe living with LOPD. These include:

- Managing the progressive nature of LOPD with the risk of irreversible damage
- The importance of individualized, holistic, multidisciplinary monitoring to enable informed, shared decision-making in an era of new and emerging treatment options. This includes consideration of emerging monitoring assessments (eg MRI) and awareness of subtle signs of progression and/or subclinical disease activity
- Making individualized treatment decisions for a patient on one treatment considering a transition to another available therapy when head-to-head data are lacking and published real-world data are limited

## Areas of Interest

Amicus is seeking grant applications for the development of an innovative, well-designed initiative that educates healthcare professionals (HCPs) on the diagnosis, management and monitoring of LOPD. Presentations and content must leverage evidence-based scientific content and be fair-balanced.

## Program Requirements

The program must be accredited and fully compliant with the criteria and/or Standards for Integrity and Independence in Accredited Continuing Education (ACCME Standards), and other relevant ethical codes and regulations.

The Policy Statement and the ACCME Standards require that a Program is conducted independently and without control or influence by Amicus Therapeutics Inc. over the Program's planning, content (including selection of speakers or moderators), or execution. The Program will also be free of commercial bias for or against any product. Amicus Therapeutics Inc. support of the Program must be clearly disclosed, including any prior relationships between Institution and Amicus Therapeutics Inc., and any prior relationships between Amicus Therapeutics Inc. and the speakers selected by the Institution.

In keeping with ACCME Standards, any product discussions in the Program must be balanced, objective, accurate and scientifically rigorous. This includes discussing the limitations of data, and that unapproved drug uses are identified as such.

The accredited provider and, if applicable, educational partner(s) must have a conflict-of-interest policy in place to identify and resolve all conflicts-of-interest from faculty or staff contributing to the development of content for this activity prior to the delivery of the program.

## Proposal Requirements

**Executive Summary:** Highlight the key elements of the program, including the elements listed below, in a one to two- page summary.

**Needs Assessment of the Gaps and Barriers:** A needs assessment independently developed and validated by the accredited provider should include an understanding of the gaps and barriers of the target audiences. The needs assessment must be referenced.

**Target Audience:** The target audience(s) for the educational program must be defined. Provide a clear rationale of why this audience is suitable to close the healthcare gap defined in the Needs Assessment.

**Audience Generation:** Describe the methods that will be employed to recruit the target audience. Include a rationale for these recruitment methods. Include information regarding the size of the recruitment audience, the number of anticipated participants and the expected number of CME/CE certificates issued.

**Learning Objectives:** Provide clearly defined and measurable learning objectives framed as practice improvements in relation to the identified barriers and gaps.

**Content Accuracy:** Describe methods to ensure complete, accurate, evidence-based review of key safety data for therapeutics discussed in the activity. Explain how the content will be updated, if necessary, throughout the program.

**Program Evaluation and Outcomes:** Provide a description of the methods to be employed and the key measurements to be assessed in evaluating this program.

**Budget:** Provide a clear breakdown of the budget (using Amicus template).

**Accreditation:** Indicate the type(s) of Continue Education credit that this program will offer (AMA, NSGC, AOA, MOC, etc.) and the name(s) of the accredited provider.

**Resolution of Conflict of Interest and Fair Balance:** Outline the practices employed by your organization to ensure that conflict of interest and fair balance of content is maintained throughout this program.

**Communication Plan:** Discuss how the provider will keep Amicus informed of program progress.

## Terms and Conditions

1. Grant applications received in response to this RFE will be reviewed in accordance with Amicus policies and guidelines.

2. All communications about this RFE must come directly to Amicus's Independent Medical Education Department via the online portal.
3. Amicus reserves the right to approve or deny applications in response to this RFE, and may cancel, in part or in its entirety, this RFE.
4. Applying for this RFE does not commit Amicus to award a grant or pay costs toward the preparation of a response to this RFE.

## References

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