

# Independent Medical Education Request for Education (RFE)

<b>Date Issued:</b>	01 May 2025
<b>RFE Requestor Information:</b>	Amicus International Medical Affairs, Pompe Disease
<b>RFE Code:</b>	RFE-PD-25-108
<b>Geographic Scope:</b>	International (ex-USA) - Priority region for the IME is Europe
<b>RFE Response Due Date:</b>	30 June 2025
<b>Amicus Submissions:</b>	Submit grant requests to: <a href="mailto:GMA_IME@amicusrx.com">GMA_IME@amicusrx.com</a>
<b>Area of Interest:</b>	Pompe Disease
<b>Educational Venue:</b>	<ul style="list-style-type: none"><li>• Entirely web-based programmes or interactive live with enduring web-based components will be considered</li><li>• Consideration will be given to in-person IME training events for HCPs at an international centre of excellence (outside of the US) for Pompe disease 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 Pompe disease (including, but not be limited to, neuromuscular specialists, geneticists, neurologists, lysosomal disease specialists, genetic counselors, pediatricians, respiratory specialists, and other healthcare professionals (such as allied healthcare specialists – specialist nurses, physiotherapists, pharmacists).
<b>Programme Budget:</b>	Single-supported and multi-supported proposals with a request amount that does not exceed \$250,000 will be considered
<b>Accreditation:</b>	CME/CPD accredited activities

## Needs Assessment:

Pompe disease is a rare, lysosomal disorder caused by pathogenic mutations in the acid alpha-glucosidase (GAA) gene leading to deficiency of the GAA enzyme and accumulation of lysosomal glycogen in all tissues with a predominant involvement of skeletal muscle.<sup>1-2</sup> The late onset form of Pompe disease (LOPD) is heterogenous, but most patients experience progressive limb-girdle weakness and respiratory insufficiency due to involvement of muscles in the proximal lower and upper limbs, paraspinal muscles, and diaphragm.<sup>1-3</sup> Clinical manifestations include difficulty walking, climbing

**One Globeside, Fieldhouse Lane,  
Marlow SL7 1HZ, United Kingdom**  
**Phone: +44 1753 888567**  
**[www.amicusrx.com](http://www.amicusrx.com)**

stairs, and progressive limitations of motor activities and activities of daily living which may ultimately require ambulatory aids and/or ventilatory assistance.<sup>1-3</sup>

Given the considerable advances in the fields' understanding of LOPD and its clinical impact, the development of new treatment options and investigational therapies and an increasing focus on shared-decision making, additional education could be beneficial to HCPs who are currently 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.<sup>3-5</sup>

### **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 programme 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 with LOPD. These include:

- The clinical implications of the heterogenous, progressive and multi-systemic nature of LOPD
- The evolving management practices for LOPD and the importance of individualized patient care that considers multidisciplinary care, holistic monitoring for disease progression (including novel methods) and shared decision-making in an era with a growing body of clinical data and new and emerging treatment options

### **Area of Interest:**

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

### **Programme Requirements:**

The programme must be accredited and fully compliant with the accrediting body's criteria and applicable government guidelines. The accredited provider and, if applicable, the medical education provider (MEP) or other third party executing the activities are expected to comply with current ethical codes and regulations. They must have a conflict-of-interest policy in place to identify and resolve all conflicts of interest from all contributors and staff developing the content of the activity prior to delivery of the programme and must have a separate company providing/accrediting independent medical education if they are also performing promotional activities.

The Programme must be conducted independently and without control or influence by Amicus Therapeutics Inc. over the Programme's planning, content (including selection of speakers or moderators), or execution. The Programme will also be free of commercial bias for or against any product. Amicus Therapeutics Inc. support of the Programme 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.

**One Globeside, Fieldhouse Lane,  
Marlow SL7 1HZ, United Kingdom  
Phone: +44 1753 888567  
[www.amicusrx.com](http://www.amicusrx.com)**



The Programme 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 programme.

**What the Proposal should include:**

**Executive Summary:** Highlight the key elements of the programme, 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 programme 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 programme.

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

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

**Accreditation:** Indicate the type(s) of Continue Education credit that this programme 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 programme.

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

**Terms and Conditions**

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

**One Globeside, Fieldhouse Lane,  
Marlow SL7 1HZ, United Kingdom  
Phone: +44 1753 888567  
[www.amicusrx.com](http://www.amicusrx.com)**



- 2) All communications about this RFE must come directly to the dedicated inbox: [GMA\\_IME@amicusrx.com](mailto:GMA_IME@amicusrx.com).
- 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.



**One Globeside, Fieldhouse Lane,  
Marlow SL7 1HZ, United Kingdom  
Phone: +44 1753 888567  
[www.amicusrx.com](http://www.amicusrx.com)**