

# Independent Medical Education Request for Education (RFE)

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| <b>Date Issued:</b>               | 9 April 2025  |
| <b>RFE Requestor Information:</b> | Amicus International Medical Affairs, Fabry Disease<br>Charlotte Sharples<br>Regional Medical Lead, International<br>E-mail: csharples@amicusrx.com   |
| <b>RFE Code:</b>                  | RFE-FD-25-107   |
| <b>Geographic Scope:</b>          | International (ex-USA)  |
| <b>RFE Response Due Date:</b>     | 30 June 2025  |
| <b>Amicus Submissions:</b>        | Submit grant requests to GMA_IME@amicusrx.com   |
| <b>Area of Interest:</b>          | Fabry 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)</li><li>• Accredited continuing medical education activities including congress events</li></ul> |
| <b>Educational Audiences:</b>     | The education should address an educational need for clinicians in the UK, EU and non-US (International) who have a role in the management of patients with Fabry disease (including, but not be limited to, cardiologists, nephrologists, geneticists, neurologists, lysosomal disease specialists, genetic counselors, paediatricians, and other healthcare professionals.)                 |
| <b>Program Budget:</b>            | Single-supported and multi-supported proposals with a request amount that does not exceed \$200,000 will be considered  |
| <b>Accreditation:</b>             | CME/CPD accredited activities   |

## Needs Assessment:

1. Increasing Accuracy of Diagnosis: Fabry disease, a lysosomal storage disorder, often presents with symptoms that overlap with those of more common conditions, such as hypertrophic cardiomyopathy

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(HCM) in cardiology. Misdiagnosis and delayed diagnosis are significant challenges; it is reported that the diagnostic delay can average 13.7 years from symptom onset. Improving differential diagnosis capabilities can significantly reduce this delay and prevent the progression of potentially irreversible complications (Ortiz, A. et al., 2021). Given these factors, there is a crucial need for educational programs that enhance clinicians' ability to distinguish Fabry disease from similar cardiac conditions, employing the latest diagnostic tools and biomarkers.

2. Underserved Populations: Fabry disease's impact on females and people of color is often underestimated due to atypical or subtler presentation of symptoms, leading to underdiagnosis and delayed treatment. Studies indicate that females with Fabry disease frequently experience a more severe progression of symptoms than previously recognized, necessitating a gender-specific approach to diagnosis and management (Germain, D.P., 2010). Similarly, there are disparities in the recognition and treatment of this disease in populations of color, highlighting a substantial gap in the current medical education pertaining to Fabry disease. Educational initiatives are needed to address these gaps and promote equitable care.

3. Changing Needs across a Lifetime: The lifelong impact of Fabry disease involves numerous psychosocial and physical challenges that evolve with age. From dealing with diagnosis and management in childhood, making informed reproductive decisions in adulthood, to managing increased cardiovascular risks in older age, each life stage requires specific knowledge and skills from healthcare providers (Politei, J. et al., 2016). Therefore, medical education that spans these various life stages, highlighting the changing needs and necessary interventions, is imperative for improving patient outcomes and quality of life.

Justification for Educational Initiatives: The outlined areas of interest address critical gaps in the current management and understanding of Fabry disease. Enhanced education in these areas can lead to earlier diagnosis, improved management strategies, and more equitable healthcare outcomes. Given the complexity of Fabry disease and its broad impact on various populations and life stages, targeted educational programs are essential. Such programs will not only raise awareness among healthcare professionals but will also equip them with the necessary tools to make significant improvements in patient care and disease 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 review of published literature, and internal Medical Affairs fair and balanced insights have established the need to address remaining practice gaps to improve the quality of care for patients with Fabry disease.

- **1. Increasing Accuracy of Diagnosis:** strategies to shorten the pathway to diagnosis and reduce misdiagnosis in Fabry disease.

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- **2. Underserved populations:** Education that serves to highlight and elevate the disease burden of underserved populations in Fabry disease
- **3. Changing Needs across a Lifetime:** Education highlighting the whole lifetime experience of living with Fabry Disease and the challenges/considerations of young people, pivotal adult life decisions

#### **Area of Interest:**

Amicus is seeking grant applications for the development of an innovative, well-designed initiative that educates healthcare professionals (HCPs) on Fabry Disease. 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 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 program, and must have a separate company providing/accrediting independent medical education if they are also performing promotional activities.

The Program must be 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.

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.

#### **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.

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**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 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 and the name(s) of the accredited provider.

**Resolution of Conflict of Interest and Fair Balance:** Outline the practices employed by your organisation 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 the dedicated inbox:  
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.

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