SANOFI Rare Disease
US Medical Affairs
Request for Proposals

Date: May 13, 2022
Disease State: Fabry Disease
Therapeutic Area: Rare Genetic Disease
Area of Interest: Fabry Disease
Geographic Scope: US

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Due Date: June 24th, 2022 by 12 PM ET
Submission Portal: https://sgrants.envisionpharma.com/vt_sgrants/
RFP Title: Fabry 2022

BACKGROUND
Fabry disease is an X-linked lysosomal storage disorder with a prevalence significantly higher than previously thought resulting from deficiency of lysosomal α-galactosidase A (GLA gene, Xq22) leading to buildup of glycosphingolipid, globotriaosylceramide (GL-3), within cells, notably endothelial cells, renal glomerular podocytes, cardiomyocytes, vascular smooth muscle cells, and neurons. Classic Fabry disease is characterized by the pediatric onset of neuropathic pain and gastrointestinal issues differentially progressing to multi-organ involvement in both males and females with a risk of severe cardiac and/or renal involvement and end stage disease in the absence of guideline-based medical management. Patients with non-classical Fabry disease may exhibit milder reductions in α-Gal A activity, a delayed onset of symptoms, slower disease progression, and a more organ-specific phenotype, in particular cardiac involvement (Hopkin et al. 2016; Ortiz et al. 2018).

FABRY CARDIAC AND KIDNEY DISEASE

Sphingolipid deposition in renal cells, including podocytes, endothelial cells, and tubular epithelial cells, leads to podocyte dysfunction and loss, focal segmental and global glomerulosclerosis, progressive chronic kidney disease associated with albuminuria/proteinuria,
and end stage kidney disease requiring dialysis and/or renal transplantation. Both baseline renal function (eGFR) and baseline level of proteinuria significantly correlate with the rate of renal function decline and renal outcomes (Schiffmann R et al. 2009; Wanner C et al. 2010). Chronic kidney disease in general is a well-established potent independent multiplier of cardiovascular risk characterized by abnormalities in endothelial function and vascular tone, progressive ventricular hypertrophy, vascular calcification, and cardiac sudden death (Go AS et al. 2004; Levey AS et al. 2011; Provenzano M et al. 2019). CKD stage 5 has been demonstrated to be an independent predictor of cardiovascular events including cardiac arrest, conduction defects, arrhythmias, and mortality in Fabry patients (Talbot AS et al. 2015). Such a relationship has been well-recognized in CKD regardless of etiology although the pathogenic mechanisms remain to be more precisely defined (Turakhia MP et al. 2018).

Clinicians face significant healthcare challenges in Fabry disease from identifying, monitoring, and addressing risk factors impacting long-term cardiac and renal disease progression and clinical outcomes to the importance of implementing evidence-based long-term disease management given these considerations (Ortiz et al. 2018; Wanner C et al. 2018).

Various organ systems including cardiac and renal potentially impacted by the Fabry disease should be thoroughly evaluated at diagnosis and overtime at appropriate intervals (Ortiz A et al. 2018). Utilizing available clinical assessment tools, imaging techniques and biochemical biomarkers have been instrumental in monitoring of disease progression. Detection of subtle organ structural and functional deterioration potentially results in improved management of disease progression.

REQUEST FOR FABRY DISEASE IME PROPOSALS
Sanofi is seeking proposals to close these independently defined healthcare gaps. Proposals can target one or multiple audiences.

- IME live/live virtual programs with or without enduring component
- Other enduring activities with national, regional and/or Local distribution channels
- Accredited or Non-accredited IME activities
- Maximum request not to exceed $250,000

Preference will be given to proposals that recommend innovative and appropriately designed interventions that are likely to enhance a learner’s knowledge of the unmet needs and employ proven strategies to overcome knowledge and performance gaps and barriers.

PROPOSALS
Proposal should include the following information
- Target Audience and Audience Generation: describe methods for reaching the target audience including description of recruitment and placement strategies to maximize participation.
- Learning Objectives and Content Accuracy: Provide clearly defined and measurable learning objectives framed as expected practice improvements in relation to the identified gaps and barriers.
- Include an overview of program content and explanation of criteria that will guide content selection, considering level of evidence and other variables. Sanofi is committed to the highest standards in ensuring patient safety; the applicant should describe methods to ensure complete, accurate, evidence-based review of key safety data for any therapeutic entities discussed in the activity. Explain how content will be updated, if necessary, throughout the program period, and how accuracy will be ensured.
• Educational Methods: Sanofi supports the ACCME guidance for educational methods to be clearly designed to address the knowledge, competence and/or performance gaps that may underlie an identified healthcare gap. Your proposal should demonstrate an understanding of instructional design as it relates to the gaps in the knowledge, competence, or performance of the targeted audience. Educational methods and design should be based on current literature in CME best practice and consistent with ACCME accreditation criteria, as applicable. Preference will be given to applications that utilize methods that have been shown to result in practice improvements, and/or with data on the effectiveness of other programs of the same type.

• Faculty Recruitment and Development: Provide Information on the expected qualifications of contributors and description of methods to ensure recruitment of course directors and faculty who meet the qualifications. Explain any methods that will be used to ensure that faculty are fully trained in the program expectations and any skills that may be needed to ensure effective delivery of intended education.

• Program Evaluation and Outcomes: Provide a description of the approach to evaluate the reach and quality of program delivery; methods for monitoring individual activities and for ensuring ongoing quality improvements. Preference will be given to programs with Objectives and Outcomes Plans with objective measures of changes in knowledge, and/or additional measures of improvements in competence, performance, patient health, population health, and/or system improvements, as aligned with the design of the intervention.

• Budget: Include a detailed budget with rationale and breakdown of costs, per unit, and description of each budget line item. In addition, please include any registrations fees paid by the learner, and how the fees will be applied. Symposium slot fee should be included and covered by the grant fees. Maximum request not to exceed $250,000.

• Accreditation: If proposal involves an accredited program, the accreditation must be provided by an appropriate accrediting body and fully compliant with the accrediting body’s criteria and applicable government guidelines and regulations.

• Fair Balance: The proposal should briefly describe methods for ensuring fair and balanced content, identification and resolution of conflict of interest, in alignment with applicable ACCME criteria.

• Communication and Publication Plan: Provide a description of how the provider will keep Sanofi informed of progress. If applicable, include description of how the results of this educational intervention will be presented, published or disseminated.

REFERENCES


Talbot AS, Lewis NT, Nicholls KM. Cardiovascular outcomes in Fabry disease are linked to severity of chronic kidney disease. Heart 2015; 101:287–293.


