

3rd SANOFI RARE DISEASES REGISTRIES *Patient Council Executive Summary*

1 April 2023

The Rare Disease Registries Patient Council met with the leadership of global and local patient advocacy groups (PAGs) to:

- *Engage in a discussion of follow-up from the 2nd Registry Patient Council, which was held in March 2022*
- *Hear from the leadership of regional and international PAGs for Gaucher, Fabry, MPS I, Pompe and ASMD diseases about changes in the real-world data ecosystem and registries*
- *Share Sanofi's priorities, focus, and activities on Rare Disease Registries patient initiatives*
- *Identify opportunities for future collaboration*

The Rare Disease Registries continue to prioritize engagement with the rare disease patient community as critical stakeholders to advance the use of real-world data in the rare ecosystem. Dissemination of the ongoing initiatives of the Registries is optimized through the collaboration with the rare disease patient advocacy community along with identification of future opportunities.

In support of the ongoing initiatives, three strategic priorities emerged from the Registries Patient Council with short- and long-term actions:

• *Increase the visibility of the Registries to stakeholders*

- Develop a plan to highlight the strengths and opportunities of the Registries
- Broaden rare disease ecosystem engagement

• *Foster the identity of the Registries Patient Council*

- Construct a communications plan to further develop its identity



• *Increase Patient Engagement in the Rare Disease Registries*

- Communication with Patient Advocacy Groups (PAGs)
 - Evolve and grow the role of the Rare Disease Registries Patient Council, including the annual face-to-face meeting and a virtual touchpoint
 - Continue patient representation at Registry Advisory Boards, expanding to regional boards in 2023
- Enable PAGs to communicate with their organizations
 - Implement digital and patient engagement initiatives and materials for increased Registry participation
 - Disseminate published plain language summaries of Registry publications while continuing to develop new ones

The Registries Patient Council identified these areas of exploration for the future:

• *Development of virtual sites to include patients without current access to Registry sites*

• *Connection of data sources including electronic medical records*

• *Exploration of how to address diversity and inclusion in the Registries*

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