RARE-X, Launches First Research Pilot Projects For Rare Disease

First global patient-powered data collection, analysis, and sharing platform will accelerate rare disease research and collaboration

Aliso Viejo, CA — Dec 2, 2020 — RARE-X, a collaborative platform for patient-controlled and structured data collection, global data sharing and analysis that will accelerate treatments for rare diseases, announces the launch of its 2021 priorities and pilots made possible by critical early-stage funding from Alexion – a leading global biopharmaceutical company focused on the development of medicines for rare diseases and devastating conditions.

The RARE-X technology platform is specifically designed to:

- **Spur the development and sharing of data sets** that aren't currently being generated, connected, properly structured and curated, or made accessible to enable meaningful research;
- **Level the playing field** for patient communities that might not otherwise have the opportunity to participate effectively in pivotal research on their behalf;
- **Expand the usability, visibility, and reach of data** from existing research programs, registries, and other aggregation efforts, thereby eliminating silos that may keep a brilliant researcher from a game-changing discovery or an innovative company from seeing the possibilities in a disease area needing a therapy;
- **Support patients in responsible data ownership and sharing**

"This data collection initiative significantly changes the outlook for patient communities needing treatments, and for researchers and drug developers working to find and develop those therapies," said Nicole Boice, RARE-X Co-Founder/Executive Director. "RARE-X positions patients everywhere to become central drivers in the efforts to progress research in their disease. RARE-X will also be supporting cross-disease research for rare conditions, as well as creating greater efficiencies that will result in a more effective and collaborative research ecosystem. A core RARE-X guiding principle is that patients own their own data, and in doing so, hold a central position in enabling research and drug discovery, to advance care and the next generation of therapies for the disease they care about."

The RARE-X platform for data contribution, aggregation, analytics, and research is powered by Broad Institute technology and expertise, which has successfully been implemented in other large-scale public data-sharing efforts. Designed to support data collection at scale, the RARE-X Data Collection Platform will have the capability to interconnect, structure, and securely accommodate a wide range of data, from genomic and clinical to patient-reported outcomes and even wearable or digital exhaust data. The types of data contributed and accessed via RARE-X will be entirely at the discretion of participating patients/patient communities and

other data owners. The goal is to help enable a vibrant ecosystem of discovery needed to support the development of future cures. The core governance principles are patient-centered and intended to meet the most stringent global standards, and have been similarly tested via other major public sector data initiatives.

"For more than 25 years, Alexion has been focused on serving patients with rare diseases and devastating conditions by developing transformative medicines, and in this time, we have made significant advances and delivered life-changing medicines to thousands of patients globally," said Wendy Erler, Vice President, Patient Experience at Alexion. "However, we recognize there is a lot more work to be done to help those living with undiagnosed rare diseases and those who still do not have a therapy today. We are proud to support the work of RARE-X, the rare disease communities, and the goal of accelerating the development of new medicines for rare diseases by empowering patients to share their data."

As RARE-X initiates its 24-month pilot period, to demonstrate how this new approach to patient-owned data collection and federated data sharing for biomedical research will accelerate disease understanding and therapy development, RARE-X will showcase the platform scalability that will efficiently support thousands of global data collection portals and patients. Key priorities include:

- Structured Data by domain – launching in Neurology, followed by four additional domain areas through the end of 2021
- Twenty (20) neurology-focused data collection portals – disease areas to be announced in early 2021
- Five (5) collaborative demonstration projects will leverage the entire technology platform supporting 21st-century data collection and federated data sharing globally—partners to be announced in early 2021.
- Scoping the first international patient owned data collection effort, with the release of a landscape report in collaboration with RARE-X founding partner, IndoUSrare.

Early RARE-X collaborators have embraced this innovative approach to transform the rare disease research landscape, including an extraordinary Board of Directors and Advisors. Other organizational collaborators include: The Alliance for AI in Healthcare, The ARM Foundation for Cell and Gene Medicine, The Broad Institute Data Sciences Division, Global Genes, Sanford Health, and additional partners being announced over the next several months.

For partnership inquiries and to support this growing effort please contact the RARE-X team at collaborators@rare-x.org.

ABOUT RARE-X™

RARE-X is a 501(c)(3) patient advocacy organization focused on supporting the acceleration and development of life-altering treatments and future cures for patients impacted by rare disease. Enabled by best-in-class technology, patients, researchers, and other technology vendors, RARE-X will gather structured, fit-for-purpose data to share broadly, benefitting from 21st-century governance, consent, and federated data sharing technology. RARE-X is building

the largest collaborative patient-driven, open-data access project for rare diseases globally. For more information, visit www.rare-x.org.

Media Contact:
Tom Hume, Marketing Communications – RARE-X
tomh@rare-x.org