Please support important rare disease FY 2022 appropriations priorities involving NIH, CDC and FDA

Request 1:
Bill: Agriculture, Rural Development, Food and Drug Administration, and Related Agencies
Section: Food and Drug Administration, Office of the Commissioner, Office of Orphan Products Development
Request: Provide $30 million for the Orphan Products Grant Program to reflect the full authorized funding level, and support the following language:

Developing Products to Treat Rare Diseases. *The Committee is aware of the increasing number of therapeutics in development for rare disease patients. As such, the Committee recognizes the importance of the Orphan Products Grant Program which supports development of products to treat orphan or rare diseases including the programs to support clinical trials and natural history studies. The Committee provides funding in this bill to increase the number of awards in both categories by ensuring the program is fully funded at $30 million to reflect the level authorized in statute, the documented burden of rare disease and the significant number of unfunded but meritorious grant applications.*

Background:

- Between 93-95% of the more than 7,000 rare diseases have no approved treatments.
- Advances in the understanding of rare diseases and novel technology platforms for therapy development mean that for many, a treatment could be within reach if the right incentives and regulatory pathways exist to make it financially viable for companies.
- Natural history studies are essential for powering rare disease therapeutic development, along with early clinical studies, but funding sources for robust natural history studies and very small population clinical research are difficult to identify.
- The FDA supports rare disease therapeutic development through the Office of Orphan Products Development and other rare disease infrastructure, including the Orphan Products Grant Program to incentivize this work in rare conditions but the amount available is severely constrained by current federal appropriation level.
- In 2020 the Orphan Products Grant Program received 47 applications but could only fund 6 grants with available funding, leaving promising science with no path forward.

Request 2:
Bill: Health and Human Services, Education, and Related Agencies
Section: Centers for Disease Control and Prevention, National Center for Health Statistics
Request: Support the following language:

Improving Accuracy of Diagnosis of Rare Diseases. *The Committee is concerned that while there are 7,000 known rare diseases, only 500 have a specific diagnostic code. This lack of a precise diagnostic code makes disease surveillance, including understanding true prevalence of a condition, more difficult and hinders diagnosis, treatment, and access to approved therapies. The Committee directs the National Center for Health Statistics at CDC to work with federal agency partners, including FDA and NIH, to establish a pathway for rare disease stakeholders to use to pursue a diagnosis code, including resources to advise stakeholders as to data and other needs and to help facilitate the application process for rare disease codes.*

Background:

- Many rare diseases do not have ICD Codes, making it hard to conduct research and understand the true disease impact.
- Without an ICD code there can be challenges in obtaining the right diagnosis and accessing treatments.
- The process to obtain ICD Codes is complex and lengthy and often rare disease patient organizations are left to advocate for codes individually.

Request 3:
Bill: Health and Human Services, Education and Related Agencies
Section: National Institutes of Health, Office of the Director
Request: Support the following language:

Rare Disease Research. *The Committee recognizes the incredible unmet need in the rare disease research community and directs NIH to prioritize funding opportunities that could lead to the evolution of science in rare diseases through work like the Rare Disease Clinical Research Network within NCATS as well as rare disease programs funded across every institute and center. The Committee further recognizes the important role the NIH-funded Undiagnosed Diseases Network (UDN) has played to improve diagnosis of rare diseases and other undiagnosed conditions and directs NIH to continue supporting this work, including by developing a plan to sustain the work of the UDN.*

Background:

- Research in rare conditions is funded across institutes at NIH.
- The National Center for Advancing Translational Sciences (NCATS) funds several large rare disease wide projects like the Rare Disease Clinical Research Network that produce results benefiting many disease areas.
- The NIH Common Fund has supported a critical program to end the diagnostic odyssey for hundreds of rare patients, the Undiagnosed Disease Network.
- Overall rare disease research across all conditions is still a small share of the NIH expenditures, and the projects supported by the Common Fund are limited to only 10 years of funding.