Testimony from Bradley Campbell, President and Chief Executive Officer, Amicus Therapeutics

"From Regulator to Roadblock: How FDA Bureaucracy Stifles Innovation"
February 26, 2026
9:30 A.M.

Chairman Scott, Ranking Member Gillibrand, and distinguished committee members:

My name is Bradley Campbell, and for the last 20 years, I've worked at Amicus Therapeutics, a Princeton, New Jersey-headquartered company whose mission is to develop and deliver transformative medicines for people living with rare diseases. Since August 2022, I have had the honor and privilege of serving as President and CEO.

I am also a member of the Board of Directors of the Biotechnology Innovation Organization (BIO), the Advisory Board of the Duke Margolis Institute for Health Policy, and the Corporate Advisory Board for the National Tay-Sachs and Allied Diseases Association.

As I speak with you today, I would note that Amicus is in the process of being acquired by BioMarin Pharmaceuticals, but my remarks today are based on my thirty years of experience in drug development, and in particular the twenty years I have spent at Amicus.

Amicus has successfully developed and commercialized three products that treat two life-threatening lysosomal storage disorders. These rare genetic disorders are caused by the body's inability to break down substances it normally would, leading to progressive, often irreversible, and potentially fatal organ and muscle damage.

These medicines are a small molecule drug chaperone for Fabry disease (Galafold), and a two-component therapy for Pompe disease that combines an oral small molecule enzyme stabilizer (Opfolda) with an infused biologic enzyme replacement therapy (Pombiliti).

I am grateful to Chairman Scott, Ranking Member Gillibrand, and the distinguished members of the Senate Special Committee on Aging for the opportunity to speak today about Amicus' experience developing medicines for serious and life-threatening rare diseases since our founding in 2002.

I am proud to speak today alongside my fellow panelists Cara O'Neill, rare disease mom and Chief Science Officer at the Cure Sanfilippo Foundation, Annie Kennedy, Chief Mission Officer at the EveryLife Foundation, and Dr. Jeremy Schmahmann, Professor of Neurology at Harvard Medical School.

I would like to thank the many members of the rare disease community who are in the room with us today, and in the Capitol this week, in celebration of Rare Disease Day, and to build on the powerful legacy of the Orphan Drug Act (ODA), passed by Congress in 1983.

Similar to the ODA, the reauthorization of the FDA's Pediatric Priority Review Voucher program on February 3 is another example of how bipartisan Congressional action, informed by

rare disease advocates, can help drive meaningful advances in rare disease research and drug development. It is critical that we protect and strengthen existing incentives for orphan drug development while we address other regulatory and policy issues.

On behalf of all of us at Amicus, I want to thank the many patients and families in the U.S. and around the world who have made our work at Amicus possible, and to share how their insights have made our work better.

For example, at a recent patient advisory group meeting, we were speaking with Pompe patients about "patient experience data," and how to make it more quantitative, which, in turn also makes these data more measurable— very salient to today's discussion of regulatory frameworks.

During a break between sessions, a young woman came up to me and said that while clinical trials in Pompe disease often measure "forced vital capacity," she depends on a mechanical ventilator to breathe. Therefore, she said, what would truly make a difference in her life was if she could just hold her breath for one minute. Why? Because if her ventilator battery fails, or she falls, or if an aide has to clear a mucus plug from her trachea tube, those 60 seconds could mean the difference between life and death.

That led all of us to reflect on how many breaths we take each day, and how easily we take for granted that the next one will always follow the last.

Her comment remains one of the most profound *and* simple examples of why it is critical to listen to the voice of patients and caregivers in designing clinical trials and regulatory endpoints that reflect what truly matters to the rare disease community.

The core message of my testimony today is straightforward and builds on this insight: the rare disease innovation ecosystem, as currently regulated, and which historically has done so much in supporting the development of new therapies for people living with rare diseases, ***must*** adapt in speed, flexibility, and scale to meet the magnitude of unmet medical need facing American patients.

We cannot ask patients to wait years for new treatments when the difference between life and death can be a single breath.

Working Together to Get Treatments to Rare Disease Patients Faster

To understand why this is true, I would like to share more about Amicus, both our successes and setbacks.

Since our founding in 2002, Amicus has grown from a small start-up into a global organization of more than five hundred team members supporting three approved medicines and the patients who rely on them.

But our success was never a given, and definitely not a straight line.

When we were developing an oral chaperone treatment for Fabry disease (Galafold), we initially planned for it to be used by all adult patients with Fabry. However, our early trials showed that

migalastat worked well for some patients, but not others. Through deeper analysis of trial data and in close dialogue with regulators, Amicus developed and validated an assay that could identify which out of the thousands of known genetic variants were responsive to treatment —and just as importantly, which were not.

The FDA ultimately incorporated this concept of "amenability" directly into the drug's labeling. The result was Galafold became the first oral precision therapy for Fabry disease, matched to patients most likely to benefit.

This is a concrete example of regulators and sponsors learning together, rather than treating trials as one-shot, binary verdicts. The result is a more convenient oral treatment option that frees a subset of Fabry disease patients from the hours-long burden of bi-weekly infusions and relieves the health care system from the added costs that come from hospital and clinic-based infusions.

The lesson is consistent: innovation is not just about creating new medicines in the lab. It requires working collaboratively with regulators, patient advocates, researchers, and more to build regulatory pathways that are flexible and designed to adapt as they learn —

providing patients and clinicians with novel treatment options as well as more data about how to apply those options to optimize health outcomes for people living with rare diseases.

But as with so many other biotechs, not every Amicus research program developing a novel medicine has crossed the proverbial finish line of FDA approval.

In epidermolysis bullosa (EB), a devastating skin disease, we launched what was at the time the largest ever trial for EB, but promising early data were contradicted in a Phase 3 trial that showed some efficacy but failed to beat placebo on the primary endpoints.

Rather than shelving the data in our archives, we communicated everything we learned with the EB community, including investigators, patient leaders, regulators, and even other companies working in the EB space.

We did this fully and deliberately, so others could build on what we had learned rather than spend scarce resources and precious patient time on ground we had already covered.

Since then, the FDA has approved the first topical gene therapy for dystrophic EB and the first cell-based gene therapy specifically for recessive dystrophic EB— proof that responsible data sharing can help move the whole rare disease community forward.

So, for Amicus, after more than 20 years in drug development, while we now have three approved drugs, we have had many more programs that were discontinued.

Rare diseases are incredibly complex and inherently more difficult to study than more common, better understood diseases. That is one reason why the vast majority of biotech companies fail, and even when they succeed, the time to get to consistent profitability and sustainability is measured in decades, not years. Amicus was founded in 2002 and became profitable only at the end of last year.

Research supported by the U.S. National Institutes of Health (NIH) estimates 95 percent of the 10,000-plus known rare diseases still lack effective, FDA-approved treatments. That means roughly 9,500 rare diseases lack treatment today.

If we keep the current pace of innovation, averaging 31 orphan-designated novel approvals per year across CDER and CBER over the last five years,[i] then developing treatments for half of all known rare diseases (~5,000 conditions) would take approximately 161 years.

We know that America's small and mid-size biotechs are the engine for new medicines overall, and for rare diseases in particular.[ii] I firmly believe that these companies will be better able to create new medicines, faster, if we adopt more flexible, agile regulatory frameworks and fully harness new tools including artificial intelligence. These approaches should also be harmonized between the U.S. FDA and other advanced regulatory agencies to minimize the collection of data that does not meaningfully advance our understanding of patient safety or product efficacy.

The FDA has reached much the same diagnosis. The creation of the Rare Disease Innovation Hub (RDIH), Rare Disease Evidence Principles (RDEP), and many other FDA programs all acknowledge that conventional drug development paradigms and evidence standards simply do not work for many rare and ultra-rare conditions.

These are all welcome efforts, but we need to transition from meetings and pilots to agency infrastructure to train staff on how to consistently implement novel approaches and best practices, convene external expert working groups to resolve scientific and regulatory bottlenecks, and de-risk promising new biomarkers.

I believe there are three fundamental areas where we need to concentrate our efforts to modernize regulations and make rapid progress without sacrificing patient safety or public health.

A. Start Clinical Trials Faster—Without Lowering Safety Standards

Other country regulators have adopted or are building faster pathways that allow early-phase trials to activate in weeks rather than months. In January 2026, the European Union launched FAST-EU (Facilitating and Accelerating Strategic Clinical Trials) to cap the time to authorization of multinational clinical trials at 70 days.[iii] Under Australia's Clinical Trial Notification (CTN) system, many trials can begin almost immediately after local ethics approval.[iv] What these models have in common is that they are shifting early clinical learning, investment, and trial leadership away from the United States. U.S. reforms should:

- Reduce administrative burdens on trial sites and sponsors by standardizing documents and processes, including clinical trial contracts, informed consent, and modular e-consent.
- Enable integration of new artificial intelligence tools to analyze complex datasets and for predictive enrollment, adaptive randomization, and site ID.
- Support single Institutional Review Boards (sIRBs), and leverage cloud services to centralize document collection and enable real-time data quality monitoring.

- Expand the use of decentralized and hybrid trials, master protocols and platform studies, RWE and external controls for decision making and remote monitoring.
- Provide Congress with routine reports on rare disease trial transformation that reflect key success metrics.

B. Use Biomarkers and Innovative Endpoints to Assess When Medicines are Working

Policymakers should support and expand FDA's capability to convene stakeholders and design fit-for-purpose biomarkers and endpoints, as well as building internal knowledge management systems that enable reviewers to apply those tools consistently and predictably. This starts with

- Sufficiently funding and staffing the Rare Disease Innovation Hub to convene more public, multistakeholder efforts that prioritize, develop, and operationalize a growing set of reusable endpoints (including composite endpoints) and biomarkers across rare diseases.
- Investing in systematic reviewer training and support so sponsors know that innovative and flexible approaches will be utilized consistently across divisions and centers.
- Harmonizing FDA and EMA expectations for high quality real-world evidence (RWE)—especially in rare pediatric populations—so it can support decisions and, where appropriate, labeling quality.
- Finalize and expand Platform Technology Designation (PTD) to enable carryover of validated assays, analytics, and chemistry and manufacturing control (CMC) elements across platform modalities (including gene therapy and gene editing), with transparent cross-center criteria and routine reporting on PTD.

C. Make Inspections and Manufacturing Rules Work Better for Rare Disease Medicines

For rare and low-volume therapies, manufacturing and inspection can become rate-limiting steps to patient access. To keep pace with innovation and the unique challenges with rare disease low volume products, FDA should:

- Expand the use of Mutual Recognition Agreements (MRAs) with other advanced regulatory agencies and expand the use of Remote Regulatory Assessments (RRAs) to reduce duplicative in-person inspections.
- Streamline foreign facility inspections by using Artificial Intelligence to prioritize risk-based inspections. This would conserve FDA inspection resources for high risk/high priority inspections and share burdens more equitably with other trusted regulatory agencies.

In addition, Congress should pass the Biomanufacturing Excellence Act of 2025 (H.R. 6089 and S. 3188) which directs the National Institute of Standards and Technology (NIST) to establish a National Biomanufacturing Center of Excellence (COE) to advance manufacturing methods to ensure innovative products can move rapidly from clinical to commercial scale.

Conclusion

Let me leave you with one final story. At an Amicus Patient Advisory Board meeting last year, one of our Fabry patient advisors said that he had stopped saving for retirement when he was first diagnosed in his late thirties. What he found online about Fabry disease at the time, was that life expectancy for men with Fabry disease was only into their late fifties.

But advances in treatment for Fabry disease, including earlier diagnosis, and better management of disease complications are extending lives and changing expectations.

That gentleman is now saving for retirement. For him, for the woman with Pompe disease who told us she needed 60 seconds to breathe, for the many patients we have learned from, and even more, around the world—we need to have a regulatory framework that can accelerate bringing these patients new treatments.

Please join me in helping to make a future to look forward to the reality for many more adults, children, and families living with rare diseases, including the advocates standing in this room today.

Thank you, and I look forward to answering your questions.

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[i] This does not include supplemental New Drug Applications, or supplemental Biological License Applications (sNDA or sBLA). Drugs with Orphan designations are exempt from the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c) requirements… It also does not include label indications for drugs that are were originally approved for non-orphan indications. While the total number of available treatments for rare diseases is higher, we believe that counting Orphan Designated approvals is the best way to measure the rate of innovation in rare disease drug development.

[ii] Biotechnology Innovation Organization. America's Innovation Engine: The Power of Small and Mid-Sized Biotechs. https://www.bio.org/sites/default/files/2026-01/the_power_of_small_and_mid-size_biotechs.pdf

[iii] https://www.aifa.gov.it/en/fast-eu

[iv] Steyn N, Davis S. *Australia: The Regulatory and Reimbursement Environment*. Third in a three-part series. Parexel; August 28, 2023. Available at: https://www.parexel.com/application/files/resources/assets/Australia%20Regulatory%20Market%20Access %20Article_Third%20in%20a%20three-part%20series%20(1).pdf (accessed Feb. 18, 2026)

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