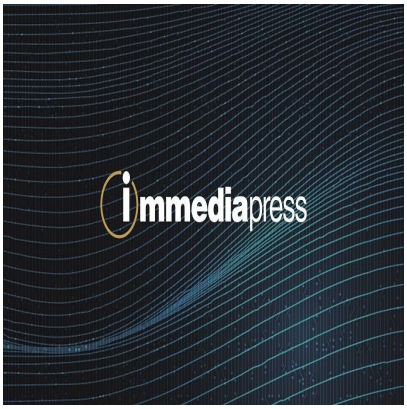

Critical Path Institute Launches â??One to Millionsâ?? to Reshape the Future of Individualized Medicine at Global Scale

Descrizione

COMUNICATO STAMPA â?? CONTENUTO PROMOZIONALE

Global initiative to scale advanced therapeutic treatments to accelerate patient access worldwide.

TUCSON, Ariz. and AMSTERDAM, March 26, 2026 /PRNewswire/ â?? Critical Path InstituteÂ® (C-Path) today announced the launch of One to Millions, a global, multi-stakeholder public-private initiative to enable scalable development of advanced therapies for highly individualized conditions. Rapid advances in technologies such as antisense oligonucleotides, genome editing, gene therapies, and RNA-based treatments are making it possible to design precisely targeted interventions for very small patient populations, even individual patients. However, existing regulatory and reimbursement frameworks, built for population-based medicines and linear development models, are not equipped to keep pace, creating a growing gap that delays patient access.

To help close this gap, One to Millions advances the U.S. Food and Drug Administration (FDA) Plausible Mechanism Framework and Rare Disease Evidence Principles by leveraging C-Pathâ??s centralized, regulatory-grade data platform to support evidence generation, regulatory decision-making, and scalable development pathways.

â??Words cannot fully express how pivotal this moment is for the transformation of lives and the long-awaited materialization of an innovative vision,â?• said Klaus Romero, M.D., MS, FCP, chief executive officer of Critical Path Institute. â??Built to make individualized therapies scalable for even more people, One to Millions is a partnership that only C-Path could convene. It features a centralized, regulatory-ready data platform; a unique precompetitive environment across the entire ecosystem; integrated preclinical, translational, clinical, and patient-level outcomes; actionable evidentiary frameworks to optimize the evaluation of efficacy and safety; and the ability to generate the regulatory-grade tools needed to establish a continuous learn-and-confirm process. There is simply no other initiative like it.â?•

A modernized platform approach brings essential consistency and reliability to advanced therapeutic technologies. Standardizing manufacturing and release-testing protocols directly addresses the inefficiencies and high costs that typically burden small-volume drug development. By building new therapies upon an established architecture, developers can leverage prior knowledge, allowing regulatory reviews to focus strictly on novel components rather than assessing the entire foundation from scratch.

Julia Vitarello, founder of Milaâ??s Miracle Foundation and co-founder of the N=1 Collaborative, emphasized the urgent need for a coordinated approach to address the shortcomings of the current landscape. â??This is a very exciting time in genetics. Today, we have the science to help a massive number of children with severe, life-altering rare diseases, but our system of access wasnâ??t designed for thousands of genetic diseases, each affecting small populations,â• Vitarello said. â??Weâ??re excited to work with regulators to move from approving one medicine for one disease at a time to building processes that can work across many diseases. This shift could be a game changer for millions of patients, but it will only succeed if we ensure continuous, iterative learning from these treatments by systematically collecting and sharing data to inform the development of safer, more effective medicines.â•

Integrating post-authorization evidence generation into the development paradigm is a defining feature of the initiative. Embedding longitudinal registries directly into the framework ensures that evidence generated for regulatory decision-making can simultaneously inform payer assessments of durability, safety, and effectiveness. Integrating information from across the ecosystem will prevent duplicative efforts and accelerate learning, a point underscored by Janet Woodcock, M.D., longtime CDER Director and prior Acting Commissioner, FDA. â??New technologies enable potential correction of the root causes of devastating monogenic diseases. But progress can stall, and regulatory requirements remain excessively conservative, when information is not available for collective analysis and learning,â• Woodcock said. â??We should not repeat the mistakes of the past; we should strive for rapid knowledge turns and agile development in this new field by sharing what we have learned. Our patients deserve no less.â•

Compiling existing preclinical, translational, and clinical data sources will help optimize toxicology and dose selection. Gathering robust data maximizes the utility of alternative methodologies, which helps reduce unnecessary reliance on animal testing while building a continuous learn-and-confirm paradigm.

â??This represents a critical new tentpole for interventional genetics, supplying a long-missing piece for approval and reimbursement and completing the arc that began with the FDAâ??s 2021 guidance on individualized antisense therapies,â• said Timothy Yu, M.D., Ph.D., Division of Genetics and Genomics, Boston Childrenâ??s Hospital and co-founder of the N=1 Collaborative. â??By effectively doubling down on modularity, this framework enables developers to leverage data across therapies targeting different genetic variants without restarting the regulatory process for each mutation. It points toward a future of â??plug-and-playâ?? genetics, but such a system cannot be built in isolation. Advancing cures that are truly greater than the sum of their parts will require shared learning through robust data sharing, where each breakthrough informs the next.â•

â??At n-Lorem, we have built a robust and scalable process to bring individualized ASO medicines to nano-rare patients. We have discovered and developed more than 25 ASOs that have enabled

treatment of more than 45 nano-rare patients to date. Many of these new ASO medicines can be used to treat more patients and we are committed to reaching these patients,â?• said Sarah Glass, Ph.D., Chief Operating Officer, n-Lorem Foundation. â??With the encouraging clinical benefit we are observing in our patients, we believe there is substantial momentum to solve some of the challenges facing the nano-rare community, to expand the accessibility of these medicines, and to find commercial solutions that will make these medicines available more broadly. We are encouraged to join the One to Millions effort and look forward to providing the insights that we have learned from the work we are conducting in the community.â?•

Participating in the collaborative effort are the n-Lorem Foundation, Milaâ??s Miracle Foundation, and the N=1 Collaborative. Founding members are coming together now. Learn more at c-path.org/programs/one-to-millions or contact partnerships@c-path.org.

View C-Pathâ??s webinar on the topic, â??Transforming Drug Development for Precision Medicines: A Practical Path from Evidence Generation,â?• to learn more, and register now for the next webinar, Wednesday, April 8, noon ET, â??Scalable Approaches to Patient-Centered Benefitâ??Risk: Defining Patient-Focused Drug Development in Precision Medicine.â?•

About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership in response to the FDAâ??s Critical Path Initiative. C-Pathâ??s mission is to lead collaborations that advance better treatments for people worldwide. Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Pathâ??s global headquarters is located in Tucson, Arizona and C-Pathâ??s Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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