CRITICAL PATH INSTITUTE

From One to Millions: Julia Vitarello on a New Vision for Individualized Medicine

“I’m very, very determined to play a role together with everyone at C-Path and others to try to make sure that the millions of kids out there like my daughter can access today’s incredible science and technology,” patient advocate Julia Vitarello said during a recent episode of the C-Path podcast, outlining her vision for how the medical system must address rare genetic diseases and scale individualized medicine.

“We’ll be here in 2,000 to 3,000 years at the pace we’re going. And that’s not acceptable,” she said while stressing the urgent need to overhaul the current clinical landscape.

Klaus Romero, C-Path CEO and host of the podcast, spoke with Vitarello about changing the infrastructure of drug development to better humanity and serve the roughly 10,000 rare genetic conditions that currently lack viable treatments. A key portion of their discussion explored C-Path’s One to Millions initiative, a collaborative effort designed to integrate data analytics platforms and build a scalable, standardized model for individualized therapies.

Vitarello confronted the severe limitations of the medical system firsthand when her daughter Mila was diagnosed with an ultra rare and fatal form of Batten disease at age six. A dedicated team at Boston Children’s Hospital were able to design a custom medicine for Mila in just one year, making her the first person in the world to receive a treatment developed for a single patient. But that timeline was an unscalable anomaly requiring a team of over 100 people, 33 separate regulatory submissions, and 3,000 pages of documentation. The intervention reduced Mila’s daily seizures but ultimately came too late. Mila passed away at age 10.

Vitarello explained that the traditional system of drug development is optimized for large populations, a model that works for a dozen common conditions. “…but the other 10,000 small or rare diseases just don’t fit in the system,” Vitarello said, as an example. “So, we can do something, we’re just kind of choosing not to.”

Vitarello presented the ideas of process approval and shared data standards as she detailed her ongoing advocacy work and could not contain her urgency about the systemic bottlenecks. Making sure the medical community accommodates this new generation of individualized therapies is absolutely critical to survival. The current framework demands that families climb an impossible administrative mountain and assemble massive, bespoke academic teams for every single dying child. “The elephant in the room” (she paused to reflect on the immense structural gaps still facing families today) “is you can’t go back to the regulator every single step of the way for every molecule for every patient.”

Vitarello explained that insisting on absolute perfection in drug development takes years, which guarantees dying children will not survive the wait. She argued for shared data to establish standards that guide developers in making medicines that are safe and effective enough for the moment. These international standards provide necessary scaffolding so parents can give informed consent and trust that developers did their absolute best with the available knowledge.

“So the technology clearly exists and the science supports that,” Romero added after Vitarello finished describing her vision of the future. Romero noted that he believes the true change for the public will emerge from collaborative data efforts like C-Path’s One to Millions initiative making that global scalable model a

reality.

The traditional drug development process is currently being challenged by new regulatory perspectives from the FDA and the UK's MHRA. Both agencies recently released frameworks acknowledging the need to group individualized medicines together to accelerate process approvals. Vitarello cautioned that regulatory approval is only a first step. Payers require standardized data demonstrating a drug's effectiveness before they will cover the costs. The current loophole in rare disease treatment forces families to rely on what Vitarello calls "cookies and lemonade" fundraising, leaving them to bear the burden of clinical costs without a predictable path to reimbursement.

"Whoever's developing these individualized medicines needs to be able to, I would say, needs to be mandated in many ways to share a certain amount of data," she told Romero. "In return, they need to be able to put a certain amount of data back into the system."

To ensure these frameworks become a reality, Vitarello continues to move beyond hypothetical models by bringing the people facing these diseases, along with drug development experience and funding, directly to regulators. Her goal is to help push those regulatory doors fully open, an effort she believes must happen in parallel with C-Path's One to Millions.

Listen to the full episode of the C-Path podcast below to hear the entirety of this urgent conversation.