The New Power in Biotech: Why Parents are Leading the Rare Disease Revolution

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The Rare Disease Innovation Crisis

Why Big Pharma stepped back, and why parents had no choice but to step up.

The "Valley of Death" is Commercial, Not Scientific

Groundbreaking ATMP Science
(Gene Therapy)

The Hurdles:
High Manufacturing Costs + Uncertain Reimbursement Models

Big Pharma & VCs Retreat

Patient Access Denied

- **High Unmet Need:** Ultra-rare diseases have devastating impacts.
- **The Business Failure:** The traditional high-volume model breaks with tiny patient populations. Payers struggle with million-dollar price tags, leading to "desinvestment" from pharma and VCs.

"We Have No Choice." The Rise of Parent-Led Foundations

- Faced with a "no" from industry, parents of children with rare and ultra-rare diseases are forming their own foundations.
- They are not just fundraising; they are de-risking the entire development process.
- Foundations like the **Cure Sanfilippo Foundation**, **Columbus Children's Foundation**, and **RTW Foundation** are now directly funding and even managing early-stage biotech programs.

De-Risking Development: The Parent & Physician Advantage

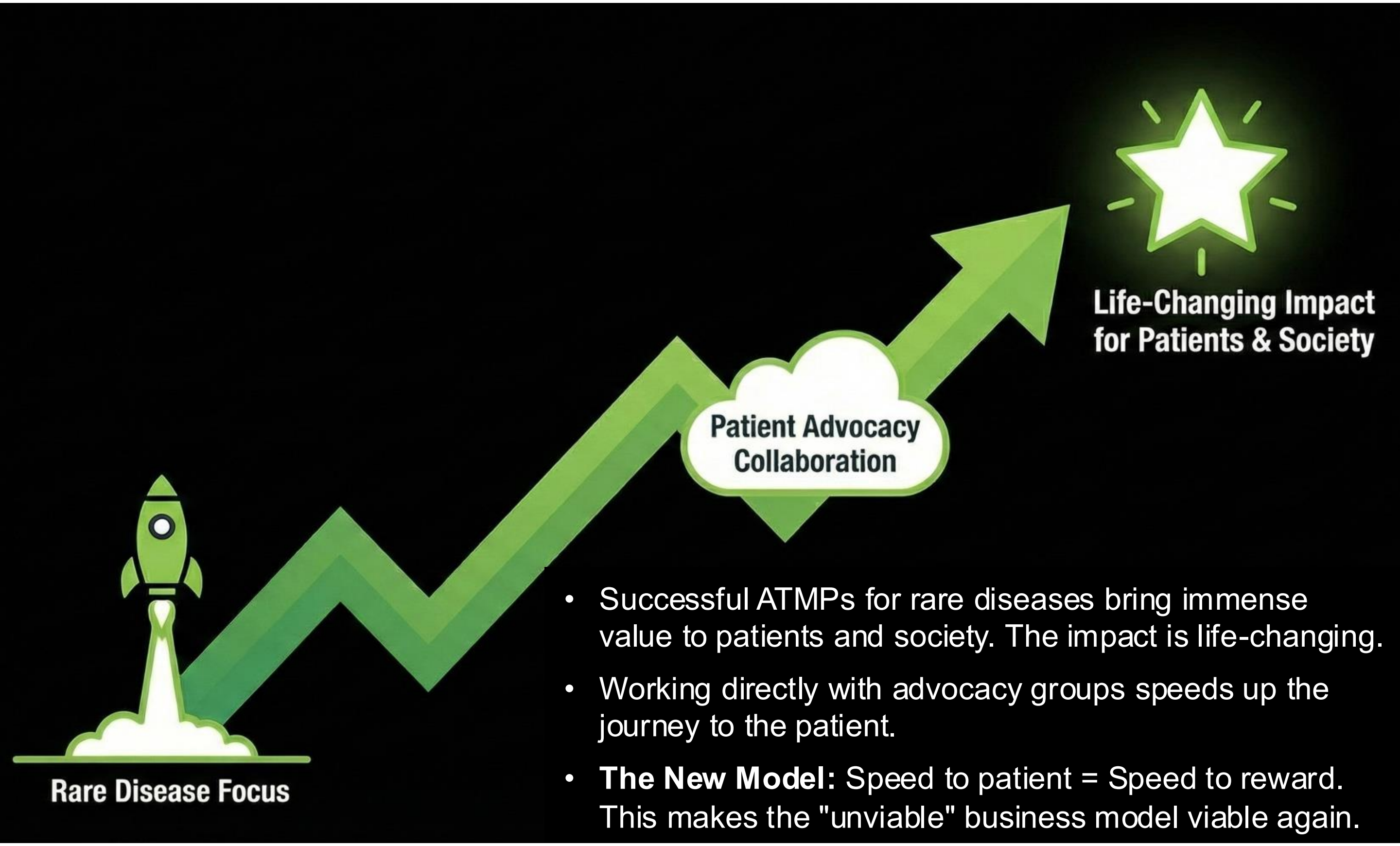
Parents: Define Real-World Endpoints (QoL)

Physicians: Connect Developers with Patients

Developers: Accelerate Clinical Trials

- **Better Endpoints:** Parents know exactly what "improvement" looks like in daily life. This creates meaningful Quality of Life (QoL) endpoints that regulators value.
- **Instant Access:** Highly specialized physicians connect developers directly to the small patient pool, making the business model viable.
- **Result:** Faster trials, better data, lower recruitment costs.

Huge Impact, Faster Reward



- Successful ATMPs for rare diseases bring immense value to patients and society. The impact is life-changing.
- Working directly with advocacy groups speeds up the journey to the patient.
- **The New Model:** Speed to patient = Speed to reward. This makes the "unviable" business model viable again.

The Future is Collaborative

Dr. Sanja Fitzgerald

- Rare disease foundations are shouldering the burden of drug development.
- As a Market Access Strategist, my role is to ensure these groundbreaking therapies don't just get approved—they get funded and reach the families who fought for them.
- **Let's connect on bridging science, advocacy, and reimbursement.**