THE NemetzGroup LLC

Rare Diseases:
Shaping Biopharma's Future from the Past

Rare Disease Continues to Be about the Patients

Patient centricity will support...

Global patient inclusion – establishing global sites enables a company to find the international community of patients and their healthcare providers. It also gleans valuable insights to improve the trial designs.

Advocacy – of the estimated 10k+ rare diseases (RareX), not all patients have organized foundations. Leverage objective, third-party organizations, like Global Genes and NORD, to support grassroots efforts to organize, engage, and enable patients to have a unified voice demanding attention.

Differentiation of treatment – incorporating patient-reported outcomes can establish patient treatment benefits beyond the regulatory endpoint requirements.

Societal benefit – demonstrating that treatment can augment living with a rare disease may empower the patients and impact the funding and policies of the system affecting their access to treatment.

Aligning with your purpose – making decisions through the patient's lens will support your credibility and integrity.

> **"Genzyme and its leaders embraced that personal sense of mission. They had a responsibility to the healthcare system as well as to the patient."**
>
> – Jim Geraghty

> **"At a company that I led, our THIRD employee was head of patient advocacy. It was that important to make sure that we built the organization around the patient and understanding their perspective."**
>
> – Paula Soteropoulos

> **"Establishing that the patient comes first stems from the CEO, the founders, and the company's mindset. The company's mission, values, and culture must convey the importance. Hire the right people who are also fully engaged and passionate about improving rare disease patients' lives."**
>
> – Masako Nakamura

> **"[On patient engagement] Do it in a genuine, open, transparent, caring way, trying to do what's right and be as honest with people and patients as possible. Many things have been learned about how to do it compliantly, appropriately, and successfully over the years from those early conversations with the FDA through approvals and advisory panel decisions."**
>
> – Jim Geraghty

Effort Is Required to Educate Policy Makers and Legislators

Engaging with policy influencers and leaders will support...

A seat at the policy table – to be part of the conversations that shape the future environment for rare disease development and access.

A revised Orphan Designation Act – the original act established in 1983 was right-sized for the period. In 2023, forty years later, new incentives, definitions, and guidelines are necessary; millions of patients deserve a renewed focus.

Redesign of the newborn screening process – to allow parents to flag risk early, provide robust testing, consistency state to state or country to country, and remove complexity. It can save lives and years of missed diagnoses. Recognition of its importance must be embraced by companies venturing to treat the 10K rare diseases.

Access to treatment – extremely high pricing for rare disease treatments is not sustainable, and change is required. Compromise among policymakers, payers, and manufacturers to ensure patients can access and afford the treatments developed for them is a shared responsibility.

Financial Markets Boom and Bust – There Is Nuance in Successful Navigation

Companies can traverse the murky market waters by...

Finding the private capital – While there are fewer IPOs and public funding mechanisms, private funding is available for companies with sound science and a focused effort on validating the biological hypothesis. Be fiscally responsible and fund what is required to achieve validation.

Leading efforts in a nascent disease – Explore rare diseases without any therapies. Be the trailblazing company to spearhead treatments for patients who have no intervention. Establish the advocacy and the clinical experts and shape their future. Biopharma investors seek novelty.

Seeking creative partnerships – nonprofits, grants, and big pharma may have funding to invest creatively in sustaining a company and its mission to validate the science and demonstrate a benefit for patients. Seeking innovative financing through strategic partnerships may provide more than just financial returns in offering expertise, expanded capabilities, and resources.

Diversifying the portfolio – Ultra-rare disease-focused companies are not a preferential investment thesis for today's investors. Communicating a strategy that leverages the ultra-rare disease as a proof of concept for a broader opportunity based on the platform or target is a valuable strategic exercise to perform.

To learn more or view our monthly e-conversations visit **nemetzgroup.com**

The NemetzGroup is a life sciences advisory firm and trusted resource. We collaborate with biopharma clients to create and implement flexible, strategic solutions across commercialization challenges. Our team of experts has a wealth of experience in the life sciences industry, and we carefully consider each engagement's unique needs and objectives. Our goal is to help companies make informed, strategic decisions that will drive success and improve the world in which we live.

Contact us at connect@nemetzgroup.com

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