# Cross-industry white paper calls for three paradigm shifts to unlock orphan drug development

*"Drive For Change" by Volv Global and industry leaders sets out a strategic roadmap to overcome the systemic barriers holding back rare disease drug development*

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ÉPALINGES, VAUD, SWITZERLAND, June 15, 2026 /EINPresswire.com/ -- In brief

- Approximately 95% of identified rare diseases have no approved treatment, with an economic burden estimated at nearly $1 trillion annually in the United States alone; Drive For Change proposes concrete paradigm shifts to address the root causes of this gap.
- The white paper identifies inaccurate population size estimates, incomplete understanding of rare disease biology, and narrow cost-burden frameworks as the three fundamental barriers to orphan drug development, and recommends specific, actionable remedies for each.
- Advanced machine learning applied to real-world patient data is identified as a critical enabling technology – capable of uncovering undiagnosed patient populations many times larger than current estimates, directly improving trial feasibility and commercial viability for pharmaceutical sponsors.

> “Throughout my career, I have seen the slow progress of novel treatments – and the root cause is the same: as an industry, we do not adequately understand the lived clinical experience of patients.”
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> *Léon van Wouwe, Clinical Innovation Director, Volv Global, Lead Author*

Volv Global today publishes Drive For Change: Paradigm Shifts and Strategic Recommendations to Overcome Barriers in Orphan Drug Development, a white paper authored in collaboration with senior leaders from pharma, Sanofi, Fondation Ipsen, Unitechpharma, and patient advocacy, brave2change. The paper is grounded in workshops and surveys conducted at

the World Orphan Drug Congress in 2023, spanning 25 countries and drawing on the perspectives of both industry professionals and patient representatives.

Despite regulatory incentives on both sides of the Atlantic, approximately 95% of the more than 10,000 identified rare diseases still have no approved treatment. Around 400 million people worldwide are affected. The diagnostic journey for rare disease patients averages close to five years, and delayed diagnosis carries a documented economic cost of up to $517,000 per patient in avoidable expenditure in the United States. At the same time, several major pharmaceutical companies have recently scaled back or withdrawn from rare disease programmes, citing high costs, scientific complexity, and uncertain returns. The pressure on the sector is intensifying at precisely the moment when patients most need it to advance.

Drive For Change argues that the underlying barriers are neither inevitable nor intractable. Three structural paradigms, the authors contend, must be challenged simultaneously: how patient populations are estimated, how rare disease biology is understood, and how the true cost and burden of disease is evaluated. On population size, the paper documents a profound gap between what practitioners believe is necessary – knowing the true prevalence of a disease – and what they believe is achievable. On disease biology, it charts the consequences of pipeline herding, where resources concentrate in well-understood indications whilst ultra-rare diseases remain largely unaddressed. On cost and burden, it demonstrates that current health technology assessment frameworks systematically undercount indirect costs, misdiagnosis expenditure, and productivity losses, resulting in orphan drugs being undervalued relative to their genuine societal impact.

Volv Global's contribution to the paper spans both conceptual framing and evidence of what is now achievable. The paper presents case study data illustrating that Volv Global's inTrigue methodology identified approximately 3.2 times the predicted patient count for a neuroendocrine tumour indication compared with the sponsor's prior epidemiological estimate, and that a prospective pilot deployment for Fabry and Pompe disease in a UK primary care setting produced a 50% to 100% increase in diagnosis rates over a period during which diagnosis rates had been unchanged for ten years. These results demonstrate that AI-driven patient-finding, applied to real-world data at population scale, can materially shift what pharmaceutical sponsors understand about addressable markets – and therefore about the investment case for orphan drug development.

The white paper reflects deliberate cross-sector authorship. Kristina An Haack, Senior Global Project Head at Sanofi and Head of Clinical Development for Inherited Neurometabolic Diseases, brings three decades of orphan drug development experience across preclinical through late-stage programmes. Professor James A. Levine, President of Fondation Ipsen, draws on thirty years of physician-scientist experience and a track record spanning more than thirty-five companies. Dr Mahdi Farhan of Unitechpharma contributes extensive expertise in drug development across biologics and small molecules, including rare diseases such as Usher syndrome. Bernd Rosenbichler, founder of brave2change and parent of a child with Alström

syndrome, provides the patient and caregiver perspective that anchors the entire paper's argument in lived experience.

From the authors
"Throughout my career in clinical development, I have seen the same barriers slow the progress of novel treatments – and time and again, the root cause is the same: as an industry, we do not adequately understand the lived clinical experience of patients, both before and after diagnosis. Today, we have more tools to do exactly that than ever before. Drive For Change is a call to use them." – Léon van Wouwe, Clinical Innovation Director, Volv Global, Lead Author

"Rare disease drug development is stuck in a self-fulfilling loop: small estimated populations, fragile business cases, programmes shelved before they reach patients. Drive For Change names the loop – and shows that with real-world data and modern AI, the addressable population is almost always larger than the sponsor believed. That is what shifts the investment case, and that is what gets treatments to patients." – Christopher Rudolf, CEO & Founder, Volv Global

"I am motivated to find and develop a treatment that makes a difference in the lives of Usher 3 patients. Every day without treatment their world becomes darker and more silent – as the loss of their most precious senses, vision, and hearing, continues. I'm inspired by these amazing individuals as they try to lead an independent life despite all the challenges." – Mahdi Farhan, Unitechpharma SA

"I watched how an undiagnosed rare disease destroyed the life of my father's beloved wife. There is no diagnosis. No cure exists. My three decades of experience as a physician-scientist supporting 37 new companies came to the fore; I can help – so can you." – Professor James A. Levine, President, Fondation Ipsen

"My son Ben's admirably positive mindset and his passion for art are what inspire me to drive for change in orphan drug development. He has been diagnosed with Alström syndrome. Watching him progressively lose his eyesight and hearing is the most difficult thing I had to face in my entire life." – Bernd Rosenbichler, Founder, brave2change

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