

# Delivering Hope: Transforming Therapies for Children with Rare Diseases

*An invited Nature Index perspective calls for innovation, sustained investment and new frameworks to transform therapies for children with rare diseases.*

DENVER, CO, UNITED STATES, July 30, 2026 /EINPresswire.com/ -- S. M.

Discovery Group (SMDG), an award-winning biotechnology company based in Denver, Colorado, United States and

Cambridge, United Kingdom is developing next-generation precision therapeutics for neurological, inflammatory and other complex diseases utilizing their proprietary platform technologies. Today the company announced the publication of an invited scientific perspective in Nature Index examining one of the greatest remaining challenges in modern medicine:



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The future of medicine depends on delivering therapies safely, efficiently and affordably to the patients who need them most.”

*Professor Moein Moghimi, Co-founder, S. M. Discovery Group*

translating advances in disease biology into safe, effective and accessible disease-modifying therapies for children living with rare diseases.

The invited Nature article, "To help children with rare diseases, we must solve drug delivery," is authored by Dr. Shadi Farhangrazi, co-founder and chief executive officer of SMDG, together with Professor Moein Moghimi, co-founder and internationally recognized pioneer in nanomedicine and targeted drug delivery. The article explores how nanotechnology, engineering biology and innovative non-viral therapeutic delivery technologies

could help overcome longstanding barriers to developing the next generation of precision medicines.

"Scientific discovery has transformed our understanding of [rare pediatric neurological diseases](#). The next challenge is translating those discoveries into therapies that are precise, safe, scalable and accessible to the patients who need them."

— Dr. Shadi Farhangrazi

Despite remarkable advances in genomics, molecular biology and precision diagnostics, effective disease-modifying therapies remain unavailable for many rare pediatric [neurological diseases](#). The authors argue that future therapeutic innovations must extend beyond identifying biological targets to include technologies capable of delivering advanced therapeutics with greater precision, improved safety, scalable manufacturing and broader patient access. While viral vectors and lipid nanoparticle (LNP)-based delivery systems have enabled important advances in genetic medicine, significant scientific and translational challenges remain, particularly for diseases requiring precise delivery to the central nervous system. These include tissue specificity, immune responses, repeat dosing, manufacturing complexity, scalability and affordability. The authors propose that continued scientific innovation, sustained investment and complementary next-generation delivery technologies will be essential to expanding therapeutic opportunities for children with rare diseases. The authors also propose a "pediatric accelerator" to support, finance and develop new therapeutics for rare neurological pediatric diseases.

The perspective highlights how SMDG's NanoLigand Carriers™ proprietary platform could enable a new generation of precision medicines for neurological diseases by delivering therapies to the right cells in the brain, improving safety, simplifying manufacturing and reducing development costs.

Professor Moein Moghimi has made pioneering contributions to targeted drug delivery, nanomedicine, lymphatic and splenic targeting, and the biological interactions and safety of advanced therapeutic systems. He has been recognized among the world's leading scientists in Pharmacology & Pharmacy in the Stanford–Elsevier global rankings. His scientific perspective, together with that of Dr. Farhangrazi, provides a forward-looking framework for translating biological discoveries into practical medicines for patients with rare diseases.

"Our vision is that tomorrow's medicines should be designed not only for efficacy, but also for precision, safety, manufacturability and accessibility. Innovation achieves its greatest impact when patients can realistically benefit from it."

— Professor Moein Moghimi

The publication reflects SMDG's mission of Developing Tomorrow's Medicines™ through proprietary NanoLigand® platform technologies and engineering biology approaches designed to translate scientific discovery into practical and affordable medicines for patients.

#### Read the Publication

The invited Nature Index perspective, "To help children with rare diseases, we must solve drug delivery," is available at:

<https://www.nature.com/articles/d41586-026-02182-4>

#### Citation

S. Farhangrazi, S. M. Moghimi (2026) To help children with rare diseases, we must solve drug delivery. Nature 655:S11

#### About S. M. Discovery Group (SMDG)

S. M. Discovery Group (SMDG) is an award-winning biotechnology company developing next-generation precision therapeutics for neurological, rare genetic, inflammatory and other complex diseases. Through its proprietary NanoLigand™ platform technologies and precision therapeutic discovery capabilities, SMDG is advancing innovative approaches for targeted therapeutic delivery and disease-modifying medicines.

#### About Professor Moein Moghimi

Moein Moghimi is the Professor of Pharmaceutics and Nanomedicine at Newcastle University, United Kingdom and Adjoint Professor, University of Colorado Anschutz Medical Campus, Denver, Colorado, United States. He is an internationally recognized pharmaceutical scientist and one of the world's leading authorities in nanomedicine, targeted drug delivery, complement biology and biomaterials. As co-founder of S. M. Discovery Group and inventor of the Company's proprietary NanoLigand™ platform technologies, Professor Moghimi has translated decades of pioneering scientific innovation into technologies designed to enable the next generation of precision therapeutics. His groundbreaking research has transformed the understanding of how nanomedicines and particulate drug delivery systems interact with biological systems, particularly the complement and innate immune systems, fundamentally advancing the rational design of safer and more effective nanomedicines. His scientific discoveries continue to underpin SMDG's mission to develop transformative therapies for neurological, rare genetic, inflammatory and other complex diseases.

#### About Dr. Shadi Farhangrazi

Shadi Farhangrazi, Chief Executive Officer and co-founder of S. M. Discovery Group, has led the Company's vision from scientific innovation toward translational medicine. Throughout her career, she has championed scientific research and patient advocacy worldwide, working to accelerate the development of transformative therapies that improve the lives of patients and their families.

#### Media Contact

Media interviews with Dr. Shadi Farhangrazi and Professor Moein Moghimi are available upon request.

Email: [admin@smdiscovery.com](mailto:admin@smdiscovery.com)

Website: [www.smdiscovery.com](http://www.smdiscovery.com)

Dr. Shadi Farhangrazi

S. M. Discovery Group (SMDG)

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