When Patients Lead:

A Blueprint for the Future of Personalized Medicine

Silvia Cerolini *– mum of Vicky*
Founder & CEO of ***Eyes on the Future***

PATIENTS as PARTNERS® EUROPE

Eyes on the Future

Vicky is 12 years old and she is going blind...

1. RDH12 Inherited Retinal Dystrophy
2. Progressive Blindness by late teens
3. No Available treatment
4. Thousands of Patients Affected Worldwide

Eyes on the Future

Over the years we have shown how patients are not just patients but are true partners

In just a few years, we have **raised millions**, built a global community and a **global patient registry**, funded **cutting-edge research**, and convened **world-leading scientists, industry and regulators**.

Leading to a gene therapy treatment that can save Vicky's sight and is ready to start clinical trials

AVV Gene therapy Proof of Concept Evidence:

- Restores enzyme activity in human cell models
- Preserves retinal function and photoreceptors in mouse models
- Demonstrates a strong preclinical safety profile
- Built on a validated platform – a similar AAV therapy is already **FDA-approved for a similar condition**

ARVO Annual Meeting Abstract | June 2022

Preparation for a Gene Augmentation Trial for *RDH12*-Associated Retinal Degenerations

Article | PDF Available

Gene Therapy for Rdh12-Associated Retinal Diseases Helps to Delay Retinal Degeneration and Vision Loss

Drug Design, Development and Therapy

August 2021 · 15:3581-3591

DOI: 10.2147/DDDT.S305378

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> Hum Gene Ther. 2019 Nov;30(11):1325-1335. doi: 10.1089/hum.2019.017. Epub 2019 Aug 5.

Development of a Gene Therapy Vector for *RDH12*-Associated Retinal Dystrophy

Kecia L Feathers [1], Lin Jia [1], Nirosha Dayanthi Perera [1], Adrienne Chen [1], Feriel K Presswalla [1], Naheed W Khan [1], Abigail T Fahim [1], Alexander J Smith [2], Robin R Ali [1, 2], Debra A Thompson [1, 3]

Affiliations + expand

PMID: 31237438 PMCID: PMC6854515 DOI: 10.1089/hum.2019.017

Even getting press coverage from around the world

Eyes on the Future

BUT the vials have been stuck on the freezer for years due to lack of commercial investment

Science is outpacing Funding and Business Models

The Traditional Model Fails Rare Diseases

- Too few patients → no commercial return.
- Traditional Clinical development too expensive
- Investors and companies walk away as not commercially attractive

Owen, UK

So we had to take our mission to the next level and take things in our own hands

Opus Genetics and the Global RDH12 Alliance Partner to Advance RDH12 Gene Therapy for Inherited Childhood Blindness

July 23, 2025 08:00 ET | Source: Opus Genetics, Inc.

Follow

Showing a new, smarter and collaborative model where industry and patients work together

Diversified financing


Life Science Leader

Patients And Industry Need A New Model For Rare Disease Gene Therapy

By Silvia Cerolini

Philanthropy + Venture

Funding & Resources

Milestone based Risk sharing

Efficiency

Lean Clinical Development: Efficient Trial Design, Manufacturing, Access and Commercial Models

Partnerships and In Kind Contribution

Innovation

New regulatory pathways for rare disease

Novel In Silico/ AI Approaches to Clinical Development

Novel AI driven endpoints

Patients driven

"This collaboration is much more than a financial arrangement. We value each other's insights, experience and connections as critical to a successful co-development of this gene therapy."

RDH12 Fund for Sight

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OPUS GENETICS

RDH12 Alliance

Sense of Urgency

Only what truly matters

Shared Resource and Governance

Our clinical trial are now FINALLY in sight…

April 06, 2026 7:00 am EDT

OPUS GENETICS

Overview | Press Releases | Events | Company Info | Financial Info | Stock Data | SEC Filings | Governance | Investor Tools

Opus Genetics plans to provide an update later in 2026 on its earlier stage programs, including OPGx-RDH12, OPGx-MERTK, and OPGx-RHO.

- **RDH12-LCA:** This program is expected to enter the clinic in the U.S. in Q4 2026. OPGx-RDH12 is the second asset licensed from Dr. Jean Bennett's lab and is partially funded through a partnership with the RDH12 Alliance to bring this program to the clinic. RDH12 is an IRD that affects children at an early age with a prevalence estimated to be 2,500 patients in the U.S. and 30,900 globally[1].

We need YOUR help to change the future of life science

Elliot, USA

- **For Vicky and our community:** save sight, see the world.
- **For rare diseases:** replicable blueprint – de-risked, capital-efficient, scalable.
- **For healthcare:** a PATIENT & INNOVATION driven path to sustainable precision medicine for ANY disease

Vicky, UK & Anna, Cyprus

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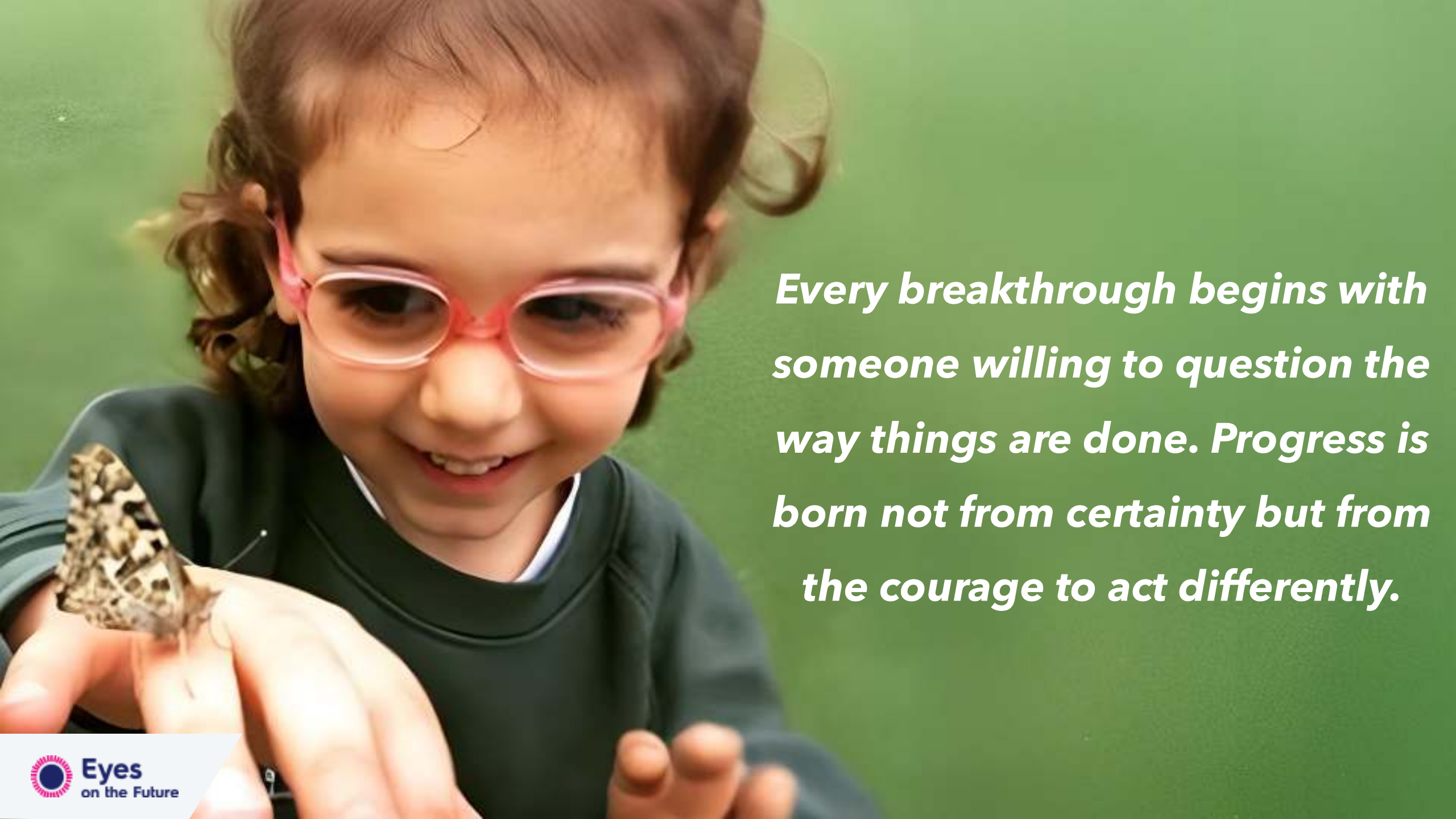

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www.eyesonthefuture.org.uk
silvia@eyesonthefuture.org.uk