Novartis International AG
Novartis Global Communications
CH-4002 Basel
Switzerland

https://www.novartis.com
https://twitter.com/novartisnews

MEDIA UPDATE

Novartis acquires Vedere Bio, adding novel optogenetic gene therapy technology for treating blindness

- *Acquisition expands the Novartis footprint in ophthalmology and enhances the company's position as an AAV-based gene therapy powerhouse.*
- *Novartis gains two pre-clinical optogenetic AAV gene therapy programs and novel delivery technology for treating inherited retinal dystrophies and geographic atrophy.*
- *Inherited retinal dystrophies, including advanced retinitis pigmentosa, affect greater than 2 million patients worldwide, and geographic atrophy affects approximately 5 million patients worldwide.*

Basel, October 29, 2020 — Novartis announced today that it has acquired Vedere Bio, adding a powerful new platform for AAV-based delivery of gene therapies and a best-in-class optogenetics program to help reimagine the treatment and prevention of vision loss and blindness. The acquisition builds on the company's commitment in cell and gene therapy, and will enable Novartis to further advance its efforts to bring transformative therapies to a wide range of patients with blinding diseases.

The technologies acquired include light-sensing proteins that can be delivered to cells in the retina and unique, adeno-associated virus (AAV) delivery vectors that enable treatment via intravitreal injection. Together, these assets have the potential to vastly expand the number of patients who could be treated for vision loss due to photoreceptor death, including all inherited retinal dystrophies.

"The next frontier in ophthalmology involves finding ways to bring potentially transformative gene therapies to a broader patient population," says Jay Bradner, President of the Novartis Institutes for BioMedical Research. "The acquisition of Vedere Bio reflects our commitment to next-generation gene therapy and brings hope to patients with otherwise untreatable forms of vision loss."

Novartis is broadening its footprint in the gene therapy space, with a focus on three distinct platforms—AAVs, chimeric antigen receptor T-cells (CAR-Ts) and clustered regularly interspaced short palindromic repeats (CRISPR). The acquisition of Vedere Bio's unique technology is the latest addition to the company's expanding therapeutic arsenal.

Inherited retinal dystrophies (IRDs) include a wide range of genetic retinal disorders marked by the loss of photoreceptor cells and progressive vision loss. The conditions, which impact more than 2 million people globally, often result in complete blindness. Yet existing treatments target only one of the more than 250 genes that can cause IRDs, limiting the population of

patients who can benefit.

The optogenetics approach acquired from Vedere Bio acts directly on surviving cells in the retina, altering their behavior and making it possible for anyone suffering from vision loss due to photoreceptor cell death to potentially benefit. This approach also holds promise for treating other conditions that involve photoreceptor loss, including a “dry form” of age-related macular degeneration (AMD) called geographic atrophy, which affects more than 5 million people globally.

The acquired optogenetics technology is based on technology from the labs of Vedere’s scientific founders, Drs. Ehud Isacoff and John G. Flannery of UC Berkeley, as well as technology directed at enhanced ocular gene therapy delivery arising jointly from UC Berkeley and the School of Veterinary Medicine at the University of Pennsylvania. It is designed to work by delivering naturally occurring, light-sensing proteins to specific retinal cells, stimulating the targeted cells to sense and transmit information to the visual processing centers in the brain. This method bypasses photoreceptor cells that may have died in retinal degeneration. Additionally, the novel AAV capsids acquired in the deal allow the optogenetic therapies to be injected intravitreally into the eye. This could potentially be administered by an ophthalmologist in the clinic.

“We believe that gene therapy technologies have transformative potential for treating blinding diseases,” said Cynthia Grosskreutz, Global Head of Ophthalmology at the Novartis Institutes for BioMedical Research. “With the new tools that this acquisition brings to the table, we will no longer be limited to replacing single genetic mutations that are causing eye diseases. This introduces the potential to treat any patient with retinal degeneration leading to photoreceptor death.”

Vedere developed the assets with the support of the investor syndicate of Atlas Venture, Mission BioCapital and Foundation Fighting Blindness (RD Fund).

Disclaimer

This media update contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as “potential,” “can,” “will,” “plan,” “may,” “could,” “would,” “expect,” “anticipate,” “look forward,” “believe,” “committed,” “investigational,” “pipeline,” “launch,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for the investigational or approved products described in this media update, or regarding potential future revenues from such products. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that the investigational or approved products described in this media update will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that such products will be commercially successful in the future. In particular, our expectations regarding such products could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases such as COVID-19; safety, quality, data

integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this media update as of this date and does not undertake any obligation to update any forward-looking statements contained in this media update as a result of new information, future events or otherwise.

About Novartis
Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 110,000 people of more than 140 nationalities work at Novartis around the world. Find out more at https://www.novartis.com.

Novartis is on Twitter. Sign up to follow @Novartis at **https://twitter.com/novartisnews**
For Novartis multimedia content, please visit **https://www.novartis.com/news/media-library**
For questions about the site or required registration, please contact media.relations@novartis.com

# # #

Novartis Media Relations
E-mail: media.relations@novartis.com

Anja von Treskow
Novartis External Communications
+41 79 392 8697 (mobile)
anja.von_treskow@novartis.com

Kara Cournoyer
Novartis Institutes for BioMedical Research
Communications
+1 617 710 5447 (mobile)
kara.cournoyer@novartis.com

Eric Althoff
Novartis US Communications
+1 646 438 4335
eric.althoff@novartis.com

Novartis Investor Relations
Central investor relations line: +41 61 324 7944
E-mail: investor.relations@novartis.com

Central		North America	
Samir Shah	+41 61 324 7944	Sloan Simpson	+1 862 778 5052
Thomas Hungerbuehler	+41 61 324 8425		
Isabella Zinck	+41 61 324 7188		