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Breakthrough technique pioneered at Novartis research institute restores light sensitivity in damaged retinas

- *The Friedrich Miescher Institute of the Novartis Research Foundation (FMI) announces a breakthrough technique that restores light sensitivity to previously unresponsive retinas*
- *Study published in the April 27 online issue of Nature Neuroscience shows that the new technique enabled blind mice to successfully perform visually guided behavioral tasks, an unprecedented result that opens the way for clinical trials in humans*
- *The approach pioneered at FMI could be an alternative to surgical transplantation of electrode arrays for the millions affected by blindness as a result of retinitis pigmentosa and late-stage macular degeneration*

Basel, 28 April, 2008 — The Friedrich Miescher Institute of the Novartis Research Foundation (FMI) today announced a novel technique that restores light sensitivity to previously unresponsive retinas in blind mice and, remarkably, produces light-induced behavioral change in mice with retinal damage. A study on the technique, published in Nature Neuroscience, indicates that the approach could be an alternative to surgical transplantation of electrode arrays.

“We are excited and encouraged by the results of the study, which open the way for clinical trials in humans,” said Susan M. Gasser, Director of FMI. “The new technique developed at the FMI could revolutionize the way that retinitis pigmentosa and late-stage macular degeneration are treated, potentially eliminating the need for surgery.”

The research team headed by Botond Roska of FMI, in collaboration with Connie Cepko of Harvard Medical School, adopted a novel approach by administering a gene of a light-activated protein from green algae to selected retinal cells (ON Bipolar cells). Their innovation involves the selective restoration of light sensitivity to circuits that respond to increases in light level (ON circuits) and not those that respond to decreasing light levels (OFF circuits). Treatment of blind mice with this technique enabled them to successfully perform visually guided behavioral tasks, an unprecedented result.

Retinitis pigmentosa is a group of inherited retinal diseases that affects about 100,000 Americans and 1.5 million people worldwide. It causes the progressive deterioration of specialized, light-absorbing cells in the retina, the paper-thin tissue that lines the back of the eye like film in a camera.*

“Contrary to common belief, the retina is a complicated organ, with more than 50 types of neurons, performing sophisticated image processing tasks,” says Botond Roska, lead

* Source: National Eye Institute, U.S. National Institutes of Health

investigator of the study at FMI. “By targeting a select population of these retinal neurons, we have been able to restore a meaningful response to light.”

Today’s research announcement is a major breakthrough in the possible treatment of one of the most devastating losses of human sensory conditions, and offers hope for treatment of a disease that frequently goes unnoticed until it has progressed past the point of treatability. Unlike other forms of retinal degeneration, retinitis pigmentosa initially inhibits night and peripheral vision, and therefore goes undetected by most afflicted with it until it has reached later stages, when it significantly limits all types of vision. The technique pioneered at FMI can also be used to treat late-stages of macular degeneration, in which photoreceptor function has been lost. Novartis already has an EU-approved treatment for patients with wet age-related macular degeneration (AMD) called Lucentis.

“This research opens a new plane of investigation, and offers hope for the millions affected by blindness due to retinitis pigmentosa and late-stage macular degeneration,” said Jose Sahel, a leading ophthalmologist in the field of retinal degeneration. “We expect these findings will become increasingly valuable as the global population ages, given that these conditions are particularly acute among the elderly. Photoreceptor restoration would be a highly desirable alternative to surgical implantation of light sensing arrays, which is the only current option for retinitis pigmentosa patients.”

Funding for the study was provided by the Novartis Research Foundation and grants from the Marie Curie Excellence program, the U.S. Office of Naval Research NICOP Grant, and Human Frontiers Science Program.

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About the FMI

The Friedrich Miescher Institute for Biomedical Research (FMI), based in Basel, Switzerland, is a world class center for basic research in life sciences. It was founded in 1970 as a joint effort of two Basel-based pharmaceutical companies and is now part of the Novartis Research Foundation. The FMI is devoted to the pursuit of fundamental biomedical research. It aims to meet the needs of modern medicine and pharmaceutical development by extending our understanding of basic molecular mechanisms that characterize human diseases. Our areas of expertise are neurobiology, growth control, which includes signaling pathways, and the epigenetics of stem cell development and cell differentiation. FMI research programmes are led by 24 staff scientists and the institute counts 320 collaborators in total. The FMI also offers training in biomedical research to PhD students and postdoctoral fellows from around the world and was designated by a survey of "The Scientist" in 2006, as the "best place for postdoctoral training" outside of the US. The quality of FMI research is internationally recognized through the large number of scientific prizes and grants awarded to its collaborators, as well as the exceptionally high citation rates of its publications. For more details see: www.fmi.ch

About Novartis

Novartis AG provides healthcare solutions that address the evolving needs of patients and societies. Focused solely on growth areas in healthcare, Novartis offers a diversified portfolio to best meet these needs: innovative medicines, cost-saving generic pharmaceuticals, preventive vaccines and diagnostic tools, and consumer health products. Novartis is the only company with leading positions in these areas. In 2007, the Group's continuing operations (excluding divestments in 2007) achieved net sales of USD 38.1 billion and net income of USD 6.5 billion. Approximately USD 6.4 billion was invested in R&D activities throughout the Group. Headquartered in Basel, Switzerland, Novartis Group companies employ approximately 98,000 full-time associates and operate in over 140 countries around the world. For more information, please visit <http://www.novartis.com>.

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Novartis Media Relations

Beatrix Benz

Novartis Global Media Relations
+41 61 324 7999 (direct)
beatrix.benz@novartis.com

Solweig Fischer

Friedrich Miescher Institute
+41 61 697 7255 (direct)
solweig.fischer@fmi.ch

e-mail: media.relations@novartis.com