Novartis International AG
Novartis Global Communications
CH-4002 Basel
Switzerland

http://www.novartis.com

MEDIA RELEASE • COMMUNIQUE AUX MEDIAS • MEDIENMITTEILUNG

Novartis receives positive CHMP opinion for Lucentis® treatment in preterm infants with retinopathy of prematurity (ROP), a disease causing visual impairment and blindness

- *Lucentis (ranibizumab), if approved in the EU for this indication, will be the first and only pharmacological therapy for ROP in these vulnerable infants*

- *Positive opinion is based on the landmark Phase III RAINBOW trial, which showed that Lucentis (ranibizumab) is an efficacious, safe and well-tolerated treatment for infants with ROP[1]*

- *Standard of care treatment for ROP, laser surgery, destroys diseased retinal tissue responsible for elevated vascular endothelial growth factor (VEGF) whereas injectable Lucentis is a pharmacological therapy that directly targets and reduces VEGF[2]*

Basel, July 26, 2019 – Novartis today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has recommended approval of Lucentis® (ranibizumab 10 mg/ml) for the treatment of preterm infants with retinopathy of prematurity (ROP). ROP is a rare eye disease but a leading cause of childhood blindness [2],[3].

The European Commission will review the CHMP opinion and is expected to deliver its final decision within three months. Lucentis, if approved for this indication, will be the first and only pharmacological therapy indicated for ROP in this vulnerable patient population. The submission is based on the randomized controlled clinical study, RAINBOW, which demonstrated that Lucentis is efficacious, safe, and well-tolerated for infants with ROP [1].

Laser surgery, the current standard of care, works by destroying the tissue in the eye that is responsible for the elevation of VEGF and can be associated with significant complications such as myopia and high myopia. While it is an effective treatment, there is a clear unmet need for innovative ways to treat ROP without destroying retinal tissue. Unlike laser surgery, Lucentis pharmacologically targets and reduces the elevated intraocular level of VEGF, the underlying cause of ROP [4],[5],[6].

If approved in the EU, Lucentis (0.2 mg dose) will be indicated for the treatment of preterm infants with zone I (stage 1+, 2+, 3 or 3+), zone II (stage 3+) or AP-ROP (aggressive posterior ROP) disease.

“Given this vulnerable patient population, and the limitations of current treatments, randomized controlled clinical studies are important to ensure safe and effective use of pharmacological therapies in pediatric patient populations. If approved, ranibizumab will be a valuable alternative treatment option to laser therapy,” commented Professor Andreas Stahl, Director of the Department of Ophthalmology, University Medical Center Greifswald.

“We recognize the importance of investing in clinical programs to secure marketing authorization ensuring safe use of pharmacological therapies in vulnerable pediatric patient populations,” commented Dirk Sauer, Development Unit Head, Novartis Ophthalmology. “We are very excited to be one step closer to bringing this transformative treatment to preterm ROP patients across Europe. The CHMP positive opinion is a testament to our relentless dedication to innovation, addressing unmet needs and reimagining eye care across all age groups.”

About Lucentis
Lucentis (ranibizumab), the first anti-vascular endothelial cell growth factor (anti-VEGF) therapy licensed for ophthalmic use, revolutionized the treatment of nAMD and has helped reduce blindness due to nAMD by 50% in several parts of the world. More than a decade of innovation and six indications (nAMD, DME, BRVO, CRVO, mCNV and other CNV) later, Lucentis continues to preserve and resolve vision for patients. We continue to investigate the possibility of Lucentis to transform the treatment of even the youngest, most vulnerable patients.

Lucentis is available in more than 110 countries and is supported by a portfolio of 251 sponsored clinical studies and extensive real-world experience. The Lucentis clinical development program has enrolled more than 130,000 patients globally across indications with 5.5 million patient-treatment years of exposure since the therapy's launch in the United States in 2006. Lucentis was developed by Genentech and Novartis. Genentech has the rights to Lucentis in the United States. Novartis has rights in the rest of the world. Lucentis is a registered trademark of Genentech Inc.

About ROP
Retinopathy of prematurity affects premature infants in both developed and developing countries. Caused by the abnormal development of retinal blood vessels in premature infants, disease progression is due to high levels of a growth factor called VEGF [6]. After premature birth, high VEGF levels can cause an infant's blood vessels in the retina to develop abnormally, which may lead to structural abnormalities such as retinal detachment, resulting in vision loss or blindness [4],[6]. The retina is the inner layer of the eye that receives light and turns it into visual messages that are sent to the brain [7]. The retina develops late in the womb and very premature babies may have incomplete development of blood vessels needed to provide oxygen [4],[6]. VEGF is an important regulator of the development of new blood vessels (known as angiogenesis) and plays a key role in the progression of ROP. The blood vessels that can develop abnormally during ROP can exert traction on the retina and lead to macular dragging, retinal detachment or other structural abnormalities resulting in vision loss or potentially blindness [6].

About RAINBOW
The RAINBOW study, which was conducted in 26 countries, is a randomized, open-label, controlled, multicenter study designed to compare the efficacy and safety of intravitreal Lucentis (ranibizumab 10 mg/ml) with laser surgery in 225 patients with ROP. The trial compared two different concentrations of Lucentis, 0.1 mg and 0.2 mg, to the current standard of care, laser surgery. The outcomes of the study were measured at 24 weeks after starting the trial. A long-term extension trial of 5 years is currently underway and expected to conclude by Q4 of 2022 [1].

About Novartis in ophthalmology
Novartis is reimagining the treatment and prevention of visual impairment and blindness. By working to push the boundaries of medicine and technology, we aim to develop life-changing gene therapies, next-generation pharmaceuticals, and transformative technologies for diseases and conditions spanning every area of eye disease, including front and back of the eye.

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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 more than 750 million people globally and we are finding innovative ways to expand access to our latest treatments. About 108,000 people of more than 140 nationalities work at Novartis around the world. Find out more at www.novartis.com.

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Novartis Global External Communications
E-mail: media.relations@novartis.com

Peter Zuest
Novartis Global External Communications
+41 79 899 9812
peter.zuest@novartis.com

Amy Wolf
Global Head Communications, Ophthalmology
+41 61 696 5894
+41 79 576 0723
amy.wolf@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	Richard Pulik	+1 862 778 3275
Pierre-Michel Bringer	+41 61 324 1065	Cory Twining	+1 862 778 3258
Thomas Hungerbuehler	+41 61 324 8425		
Isabella Zinck	+41 61 324 7188		