

## **FDA Awards Isarna Orphan Drug Designation for ISTH0036 to Improve Glaucoma Treatment Outcome**

**Munich, Germany, June 10, 2015** – [Isarna Therapeutics](#), the leader in transforming growth factor beta (TGF- $\beta$ ) isoform targeted antisense therapeutics, today announced that the Food and Drug Administration (FDA) has granted orphan drug designation for ISTH0036, a locked nucleic acid-modified antisense oligonucleotide, for the prevention of scarring post glaucoma filtration surgery. The FDA decision follows recently announced EMA orphan drug designation for ISTH0036, which is currently in Phase I clinical evaluation in glaucoma patients.

“Both EMA and FDA decisions support the innovation required to improve standard of care for advanced-stage glaucoma patients. Isarna continues to gather insight into the molecular role played by TGF- $\beta$ 2 in glaucoma pathophysiology and we value the recognition from both agencies of ISTH0036’s potential,” said Dr. Philippe Calais, President and Chief Executive Officer of Isarna Therapeutics.

Glaucoma filtration surgery is the last line of treatment for patients with advanced-stage glaucoma. Although well established as a procedure to lower intraocular pressure and prevent further deterioration of the visual field, scarring counteracts the positive effect of the surgery. ISTH0036 is currently the only compound in clinical development worldwide that directly targets TGF- $\beta$ 2, a core driver of glaucoma pathophysiology. In addition to glaucoma, several other diseases in ophthalmology have been linked to the modulation of TGF- $\beta$ , including diabetic retinopathy, proliferative vitreoretinopathy, and several corneal diseases.

Under the US Orphan Drug Act (ODA), orphan drug designation provides seven-year exclusive marketing period for that drug in addition to certain incentives and tax credits for development costs. More information on the ODA and the FDA Office of Orphan Products Development can be located at <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm>.

### **About Glaucoma**

Glaucoma is a progressive optic neuropathy that is the leading cause for irreversible blindness worldwide. The disease has been linked to elevated intraocular pressure, due to decreased fluid outflow (aqueous humor) from the eye, based upon alteration of the trabecular meshwork. Recent scientific data indicate that glaucoma progression is associated with elevated levels of TGF- $\beta$ 2 resulting in alteration of the trabecular meshwork (Prendes et al. 2013; Br J Ophthalmol.) and a potential direct pathophysiologic effect on the optic nerve (Fuchshofer 2011; Exp Eye Res.). Approximately 10% of glaucoma patients lose vision despite optimum treatment. More information on glaucoma can be found at [www.glaucoma.org](http://www.glaucoma.org), a website of the Glaucoma Research Foundation.

### **About ISTH0036**

ISTH0036 is a locked nucleic acid-modified antisense oligonucleotide selectively targeting the messenger ribonucleic acid (mRNA) of TGF- $\beta$ 2. TGF- $\beta$  (transforming growth factor beta) plays an important role in key pathways such as cell proliferation, cell differentiation, immune response and tissue modeling. Because TGF- $\beta$  is chronically elevated in many diseases, including ophthalmic and fibrotic diseases and cancer, and involved in their pathophysiology, it is an extremely versatile drug target throughout the body. Preclinical studies have demonstrated that ISTH0036 is highly potent and shows selective target engagement (TGF- $\beta$ 2 mRNA and protein downregulation) and long-lasting tissue uptake and pharmacodynamic effects.

### **About Isarna Therapeutics**

Isarna Therapeutics has an unmatched commitment to developing selective TGF- $\beta$  inhibitors to effectively treat ophthalmic and fibrotic diseases and fight cancer. We are advancing a unique pipeline of novel oligonucleotides and combination modalities to transcend clinical response and improve patient outcomes. Isarna is established in the Netherlands, Germany, and the United States. [www.isarna-therapeutics.com](http://www.isarna-therapeutics.com).



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