

Roche's Port Delivery System with ranibizumab shows positive phase III results in neovascular age-related macular degeneration

- **Port Delivery System with ranibizumab (PDS) is a permanent refillable eye implant that continuously delivers ranibizumab over a period of months, potentially reducing the treatment burden associated with frequent eye injections**
- **Refilled every six months, PDS demonstrated non-inferior and equivalent efficacy compared to the standard of care – monthly ranibizumab eye injections – for people with neovascular age-related macular degeneration**
- **Data from the Archway study will be presented at an upcoming medical meeting and submitted to health authorities around the world**

Basel, 27 May 2020 - Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced positive topline results from the phase III Archway study, evaluating Port Delivery System with ranibizumab (PDS) in people living with neovascular or “wet” age-related macular degeneration (nAMD). PDS is a permanent refillable eye implant, approximately the size of a grain of rice, which continuously delivers a customised formulation of ranibizumab over a period of months. The Archway trial met its primary endpoint, demonstrating that patients with PDS who received refills every six months achieved visual acuity outcomes equivalent to those receiving monthly ranibizumab 0.5 mg injections. In Archway, PDS was generally well-tolerated with a favorable benefit-risk profile.

Neovascular AMD is a leading cause of blindness in people aged 60 and older globally. ^[1,2] The current standard of care for nAMD requires patients to visit their ophthalmologist as often as monthly for eye injections of anti-vascular endothelial growth factor (VEGF) therapy to help maintain vision gains and/or prevent vision loss. ^[3] This high treatment burden with anti-VEGF therapy can lead to under-treatment of nAMD and, potentially, less than optimal vision outcomes. ^[3,4]

“For people around the world receiving frequent eye injections for neovascular AMD, this continuous delivery system could greatly reduce their treatment burden,” said Levi Garraway, M.D., Ph.D., Roche’s Chief Medical Officer and Head of Global Product Development. “We look forward to presenting detailed Archway results at future medical meetings and discussing these data with regulatory authorities, with the aim of bringing this new treatment option to patients as soon as possible.”

In addition to Archway, the Portal study is investigating the long-term safety and tolerability of PDS for the treatment of nAMD. ^[5] Furthermore, PDS is also being studied in the Pagoda trial for the treatment of diabetic macular edema (DME), a vision-threatening complication of diabetes. ^[6]

Full results from the Archway study will be presented at an upcoming medical meeting and submitted to health authorities around the world, including the US Food and Drug Administration and European Medicines Agency, for consideration of regulatory approval for the treatment of nAMD.

About the Archway study [7]

Archway (NCT03677934) is a randomised, multicentre, open-label phase III study evaluating the efficacy and safety of Port Delivery System with ranibizumab (PDS), refilled every six months at fixed intervals, compared to monthly intravitreal injections of ranibizumab 0.5 mg, in 418 people living with neovascular age-related macular degeneration. The primary endpoint of the study is the change in best-corrected visual acuity (BCVA) score (the best distance vision a person can achieve – including with correction such as glasses – when reading letters on an eye chart) from baseline at the average of week 36 and week 40. Secondary endpoints include: safety; overall change in BCVA from baseline; and change from baseline in center point thickness over time.

About neovascular age-related macular degeneration

Age-related macular degeneration (AMD) is a condition that affects the part of the eye that provides sharp, central vision needed for activities like reading. Neovascular or “wet” AMD (nAMD) is an advanced form of the disease that can cause rapid and severe vision loss. It develops when new and abnormal blood vessels grow uncontrolled under the macula, causing swelling, bleeding and/or fibrosis. [8] Worldwide, around 17 million people are living with nAMD – the leading cause of vision loss in people over the age of 60 – and the disease will affect even more people around the world as the global population ages. [1,2,9]

About Port Delivery System with ranibizumab

Port Delivery System with ranibizumab (PDS) is a permanent refillable eye implant, approximately the size of a grain of rice, which is designed to continuously release a customised formulation of ranibizumab into the eye over time. Ranibizumab is a vascular endothelial growth factor (VEGF) inhibitor designed to bind to and inhibit VEGF-A, a protein that plays a critical role in the formation of new blood vessels and the leakiness of the vessels. [10] PDS contains a customised formulation of ranibizumab not approved by regulatory authorities. It is different from the ranibizumab intravitreal injection, a medicine marketed as Lucentis®* (ranibizumab injection) which is approved to treat neovascular age-related macular degeneration (nAMD) and other retinal diseases. [11]

By maintaining therapeutic drug concentration levels of ranibizumab with two refills per year, PDS may offer greater outcomes certainty in terms of vision gains and maintaining those gains for people living with nAMD. Additionally, by decreasing the need for frequent injections and physician visits, PDS may reduce the burden of treatment associated with standard anti-VEGF treatments. [3] The Archway trial of PDS in nAMD is evaluating a regimen of PDS implantation followed by twice yearly refills.

About Roche in Ophthalmology

Roche is focused on saving people's eyesight from the leading causes of vision loss through pioneering therapies. Through our innovation in the scientific discovery of new potential drug targets, personalised healthcare, molecular engineering, biomarkers and continuous drug delivery, we strive to design the right therapies for the right patients.

Applying our extensive experience, we have already brought breakthrough ophthalmic treatments to people living with vision loss through Lucentis** (ranibizumab injection) in 2006, the first treatment approved to improve vision in people with certain retinal diseases, including neovascular age-related macular degeneration (nAMD), diabetic macular edema (DME), diabetic retinopathy (DR), retinal vein occlusion (RVO) and myopic choroidal neovascularisation. [11]

With a robust pipeline, rationalised in science and informed by insights from people with eye diseases, Roche has the broadest late stage retina pipeline including treatments for nAMD, DME, DR and RVO. Our early stage pipeline also includes gene therapies and treatments for geographic atrophy and other vision-threatening diseases, including rare and inherited conditions. [12]

About Roche

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people's lives. The combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management.

Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. More than thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Moreover, for the eleventh consecutive year, Roche has been recognised as one of the most sustainable companies in the Pharmaceuticals Industry by the Dow Jones Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2019 employed about 98,000 people worldwide. In 2019, Roche invested CHF 11.7 billion in R&D and posted sales of CHF 61.5 billion. Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit www.roche.com.

*Lucentis® (ranibizumab injection) was developed by Genentech, a member of the Roche Group. Genentech retains commercial rights in the United States and Novartis has exclusive commercial rights for the rest of the world.

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