USE OF COMPOUNDS THAT INTERFERE WITH THE HEDGEHOG SIGNALING PATHWAY FOR TREATING OCULAR DISEASES RELATED WITH NEOVASCULARIZATION

Fondazione Telethon, a major Italian charity, has developed new methods for preventing, inhibiting, and/or reversing ocular neovascularisation in mammalian subjects by interfering with the Hedgehog signaling pathway. Ocular neovascularisation is responsible for many diseases, including age-related macular degeneration, (proliferative) diabetic retinopathy, neovascular glaucoma, retinal vein occlusion, retinopathy of prematurity. Partners should develop drugs/compounds/vector-based gene delivery systems for the treatment of the above-mentioned diseases.

Description:
The present invention relates to methods, and more specifically to the use and administration to a mammalian subject of a therapeutically effective amount of compounds that interfere with Hedgehog signalling for preventing, inhibiting, and/or reversing ocular neovascularisation. Ocular neovascularisation is a major cause of blindness in developed countries and it is causally involved in many ocular diseases such as age-related macular degeneration (AMD), (proliferative) diabetic retinopathy, neovascular glaucoma, retinal vein occlusion, or retinopathy of prematurity (ROP). Current treatments are of limited efficacy and associated with significant adverse effects, reflecting the high unmet need in those disease areas.

The inventors have shown in an animal model that interference with Hedgehog signaling by means of cyclopamine is able to prevent, inhibit and/or reverse ocular neovascularisation, and that such treatment has a therapeutic effect. Upon administration of a Hedgehog signaling interference substance, such as a protein or a siRNA, ocular neovascularisation will be prevented, inhibited and/or reversed, thus preventing and/or ameliorating the pathologies associated with such condition.

Innovative Aspects of the offer
This invention could be employed for the manufacturing of a medicament useful in preventing, inhibiting, and/or reversing ocular neovascularisation-associated diseases. Such drug (protein, siRNA) could be delivered both as a peptide or as a vector-based gene transfer system.

Main Advantages of the offer
Laser photocoagulation is the current treatment for ocular neovascularisation, slowing the neovascularisation process, but at the same time severely damaging the retina. Administration of a compound / peptide / gene would inhibit ocular neovascularisation without resulting in the laser-induced retinal damage.

Task to be performed by the partner
Develop, manufacture and distribute gene and molecular therapy for ocular neovascularisation diseases. Clinical trials.

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References:
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