protein underlying a loss-of-function disease. In some aspects, the amyloid-forming protein is selected from the group consisting of Hsp27, αA-crystallin, αB-crystallin, βB2-crystallin, βB1-crystallin, γD-crystallin, Hsp22, Hsp20, tau, Alpha-synuclein, IAPP, beta-amyloid, PrP, Huntingtin, Calcitonin, Atrial natriuretic factor, Apolipoprotein AI, Serum amyloid A, Medin, Prolactin, Transthyretin, Lysozyme, Beta 2 microglobulin, Gelsolin, Keratoepithelin, Cystatin, Immunoglobulin light chain AL, and S-IBM. In other aspects, the protein underlying a loss-of-function disease is selected from the group consisting of mutant β-glucosidase, cystic fibrosis transmembrane receptor, hexosaminidase A, hexosaminidase B, β-galactosidase, and alpha-glucosidase.

[0010] Other features and advantages of the present disclosure will become apparent from the following detailed description. It should be understood, however, that the detailed description and the specific examples, while indicating specific embodiments of the disclosure, are given by way of illustration only, because various changes and modifications within the spirit and scope of the disclosure will become apparent to those skilled in the art from this detailed description. The entire document is intended to be related as a unified disclosure, and it should be understood that all combinations of features described herein are contemplated, even if the combination of features are not found together in the same sentence, or paragraph, or section of this document. In addition to the foregoing, the invention includes, as an additional aspect, all embodiments of the invention narrower in scope in any way than the variations specifically mentioned above. For example, if aspects of the invention are described as "comprising" a feature, embodiments also are contemplated "consisting of or "consisting essentially of" the feature.

[0011] In one embodiment, this invention discloses the use of a composition for the preparation of medicament to treat and/or prevent vision disorders in a subject, said composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol. Said subject is having or at risk of developing a vision disorder that affects the normal structure of the lens in the eye. Said subject may be selected from the group consisting of amphibians, reptiles, avians, and mammals; wherein said mammal may be selected from the group consisting of rodents, cats, dogs, pigs, horses and humans. In another embodiment, said vision disorder is selected from the group consisting of cataract, congenital cataracts, cortical opacities, posterior subcapsular cataract, presbyopia nuclear sclerosis, retinal degenerative disorder, Refsum disease, Smith-Lemli-Opitz syndrome, Schnyder crystalline corneal dystrophy, drusen, age-related macular degeneration, and diabetic retinopathy, and lanosterol inhibits crystallin protein aggregation.

[0011a] In one embodiment, this invention discloses the use of a composition in the manufacture of medicament to treat and/or prevent vision disorders in a subject in need thereof, said composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol, and wherein said vision disorder is selected from the group consisting of cataract, congenital cataracts, cortical opacities, posterior

subcapsular cataract, presbyopia nuclear sclerosis, retinal degenerative disorder, Refsum disease, Smith-Lemli-Opitz syndrome, Schnyder crystalline corneal dystrophy, drusen, age-related macular degeneration, and diabetic retinopathy, wherein said composition is to be administered to an eye of said subject, wherein said subject is having or at risk of developing a vision disorder that affects the normal structure of the lens in the eye.

[0012] In yet another embodiment, this invention discloses the use of a composition for the preparation of medicament to treat cataract or blindness/impaired vision in a subject, said composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol, wherein said lanosterol dissolves lens crystallin protein aggregate(s) in the eye of said subject; wherein the lens crystallin protein is any of α–crystallin, β–crystallin or γ–crystallin. The above mentioned composition may be formulated as an ophthalmic solution, an ophthalmic ointment, an ophthalmic wash, an intraocular infusion solution, a wash for anterior chamber, an internal medicine, an injection, or preservative for extracted cornea.

[0012a] In yet another embodiment, this invention discloses the use of a composition in the manufacture of medicament to treat cataract or blindness/impaired vision in a subject in need thereof, said composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol, wherein said composition is to be administered to an eye of said subject, wherein said lanosterol dissolves lens crystallin protein aggregate(s) in the eye of said subject, wherein said cataract or blindness/impaired vision in said subject is a vision disorder that affects the normal structure of the lens in the eye.

[0013] In yet another embodiment, this invention discloses a method for dissolving amyloid-like fibrils of crystallin proteins, comprising the step of contacting the amyloid-like fibrils with lanosterol in a sufficient amount and duration so as to dissolve the amyloid-like fibrils of crystalline proteins, wherein the method may be performed in situ, in vitro or in vivo. The method may be performed on a subject selected from the group consisting of amphibians, reptiles, avians, and mammals; wherein said mammal may be selected from the group consisting of rodents, cats, dogs, pigs, horses and humans.

[0013a] In yet another embodiment, this invention discloses an *in vitro* method for dissolving amyloid-like fibrils of crystallin proteins, comprising the step of contacting the amyloid-like fibrils with lanosterol in a sufficient amount and duration so as to dissolve the amyloid-like fibrils of crystalline proteins.

[0014] In another embodiment, this invention discloses a kit for treating and/or preventing vision disorders that affect the normal structure of the eye in a subject, comprising a formulation of a pharmaceutically effective amount of lanosterol, a pharmaceutically acceptable carrier and instructions for administering said formulation such that said administration treats and/or prevents said vision disorder. In yet another embodiment, this invention discloses an ophthalmic pharmaceutical composition for treating and/or preventing vision disorders in a subject, said

composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol; wherein said composition may be formulated as an ophthalmic solution, an ophthalmic ointment, an ophthalmic wash, an intraocular infusion solution, a wash for anterior chamber, an internal medicine, an injection, or preservative for extracted cornea.

[0015] In another embodiment, this invention discloses a method for identifying and/or treating a subject at risk of developing cataract or blindness/impaired vision associated with formation of lens crystallin protein aggregate(s) in an eye, comprising: a) assaying for amount of lanosterol synthase activity in the subject; b) determining whether the amount of lanosterol synthase activity is less than that of a control population.

CLAIMS

What is claimed is:

1. Use of a composition in the manufacture of medicament to treat and/or prevent vision disorders in a subject in need thereof, said composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol, and wherein said vision disorder is selected from the group consisting of cataract, congenital cataracts, cortical opacities, posterior subcapsular cataract, presbyopia nuclear sclerosis, retinal degenerative disorder, Refsum disease, Smith-Lemli-Opitz syndrome, Schnyder crystalline corneal dystrophy, drusen, age-related macular degeneration, and diabetic retinopathy, wherein said composition is to be administered to an eye of said subject, wherein said subject is having or at risk of developing a vision disorder that affects the normal structure of the lens in the eye.

2. The use of claim 1, wherein said lanosterol dissolves lens crystallin protein aggregate(s) in the eye of said subject.

3. The use of claim 1, wherein said vision disorder is cataract.

4. The use of claim 1, wherein said lanosterol inhibits crystallin protein aggregation.

5. Use of a composition in the manufacture of medicament to treat cataract or blindness/impaired vision in a subject in need thereof, said composition comprises a pharmaceutically acceptable ophthalmic carrier and a pharmaceutically effective amount of lanosterol, wherein said composition is to be administered to an eye of said subject, wherein said lanosterol dissolves lens crystallin protein aggregate(s) in the eye of said subject, wherein said cataract or blindness/impaired vision in said subject is a vision disorder that affects the normal structure of the lens in the eye.

6. The use of claim 4, wherein the lens crystallin protein is selected from the group consisting of α-crystallin, β-crystallin, γ-crystallin, and combination thereof.

7. The use of any one of claims 1-6, wherein said composition is formulated as an ophthalmic solution, ophthalmic gel, an ophthalmic ointment, an ophthalmic wash, an intraocular infusion solution, a wash for anterior chamber, an internal medicine, an injection, or preservative for extracted cornea.

8. The use of any one of claims 1 or 3-6, wherein said subject is selected from the group consisting of amphibians, reptiles, avians, and mammals.

9. The use of claim 8, wherein said mammal is selected from the group consisting of rodents, cats, dogs, pigs, horses and humans.

10. The use of any one of claims 1-6, wherein said composition is formulated for topical, subconjunctival, retrobulbar, periocular, subretinal, suprachoroidal, or intraocular administration.

11. The use of any one of claims 1-6, wherein said composition is formulated for topical administration.

12. The use of any one of claims 1-6, wherein the pharmaceutically acceptable ophthalmic carrier is selected from water, a buffer, or a solution of sodium chloride.

13. The use of any one of claims 1-6, wherein the pharmaceutically acceptable ophthalmic carrier is a gel.

14. The use of any one of claims 1-6, wherein the pharmaceutically acceptable ophthalmic carrier is an ointment.

15. The use of any one of claims 1-6, wherein the pharmaceutically acceptable ophthalmic carrier is a cyclodextrin.

16. An *in vitro* method for dissolving amyloid-like fibrils of crystallin proteins, comprising the step of contacting the amyloid-like fibrils with lanosterol in a sufficient amount and duration so as to dissolve the amyloid-like fibrils of crystalline proteins.