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(54) Title: METHODS, ASSAYS AND COMPOSITIONS FOR TREATING RETINOL-RELATED DISEASES

(57) Abstract: Described herein are methods and compositions for treating certain retinol-related diseases and conditions by modulation of transthyretin (TTR) and retinol binding protein (RBP) availability in the subject. For example, the methods and compositions provide for therapeutic agents for the treatment and/or prevention of age-related macular degeneration and/or dystrophies, metabolic disorders, idiopathic intracranial hypertension, hyperostosis, and protein misfolding and aggregation diseases. The compositions disclosed may be used as single agent therapy or in combination with other agents or therapies. In addition, described herein are methods and assays for selecting appropriate agents that can modulate the TTR and RBP availability in a subject.

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