

RETINA PIPELINE 2023

A VIEW INTO ONGOING INNOVATION

DRY AMD

1 Suppress Inflammation

Targeting the Complement Cascade

- 1 **ANX007** (Annexon) – C1q Inhibition
- 2 **Pegcetacoplan** (Apellis) – C3 Inhibition
- 3 **CB-2782-PEG** (Catalyst Biosciences) – C3 Protease
- 4 **NGM621** (NGM Biotherapeutics) – C3 Inhibition
- 5 **AMY106** (Amyndas Pharmaceuticals) – C3 Inhibition
- 6 **SLN501** (Silence Therapeutics/Mallinckrodt Pharmaceuticals) – siRNA vs C3
- 7 **PRO657** (Mosaic/Ocular Therapeutix) – C3 Inhibition
- 8 **iPTACOPAM** (Novartis) – Bb Inhibition
- 9 **AGN-151151** (AbbVie) – C5 Inhibition
- 10 **cEMDISIRAN** (Alnylam Pharmaceuticals) – siRNA C5 mRNA Inhibitor
- 11 **KNP-302** (Kanaph Therapeutics) – C3b and CD59 Inhibition
- 12 **Avacincaptad pegol** (Iveric Bio) – C5 Inhibition
- 13 **ALXN1720** (Alexion) – C5 Inhibition
- 14 **PASylated Nomacopan** (Akari Therapeutics) – Bispecific Inhibition of C5 & LTβ4
- 15 **JNJ-1887** (Janssen) – MAC Inhibition Gene Therapy
- 16 **Ionis-FB-LRx** (Ionis/Roche) – Complement Factor B Inhibition
- 17 **ALXN2040** (Alexion Pharmaceuticals/AstraZeneca) – Complement Factor D Inhibition
- 18 **BCX9930** (BioCryst Pharmaceuticals) – Complement Factor D Inhibition
- 19 **SFH GTx** (Aevitas Therapeutics) – Complement Factor H Gene Therapy
- 20 **CFH GTx** (Syncona) – Complement Factor H Gene Therapy
- 21 **GEM103** (Gemini Therapeutics) – Recombinant Complement Factor H Therapy
- 22 **AAV.SFH** (Aevitas) – Short-Form Complement Factor H Gene Therapy
- 23 **AVD-104** (Aviceda Therapeutics) – Complement Factor H Activation
- 24 **CB-4332** (Catalyst) – Recombinant Complement Factor I
- 25 **PPY 988** (Gyroscope Therapeutics/Novartis) – Complement Factor I Gene Therapy
- 26 **OMS721** (Omeros) – Blocks MASP-2
- 27 **OMS906** (Omeros) – Blocks MASP-3
- 28 **KNP-301** (Kanaph Therapeutics) – Blocks C3b/Anti-VEGF
- 29 **IBI302** (Innovent Biologics) – sCR1 Inhibition/Anti-VEGF
- 30 **NM5072** (NovelMed) – Properdin Inhibition
- TMI-018** (Translatum Medicus Inc) – Blocks M1 Transcriptome Expression

2 Reduce Toxic By-Product Accumulation

Prevents Amyloid Aβ Oligomer Assembly

- GAL-101** (Galimedix Therapeutics/lacta)
- ALZ-801** (Alzheon)
- VUTRISIRAN** (Alnylam Pharmaceuticals) – siRNA TTR Silencer

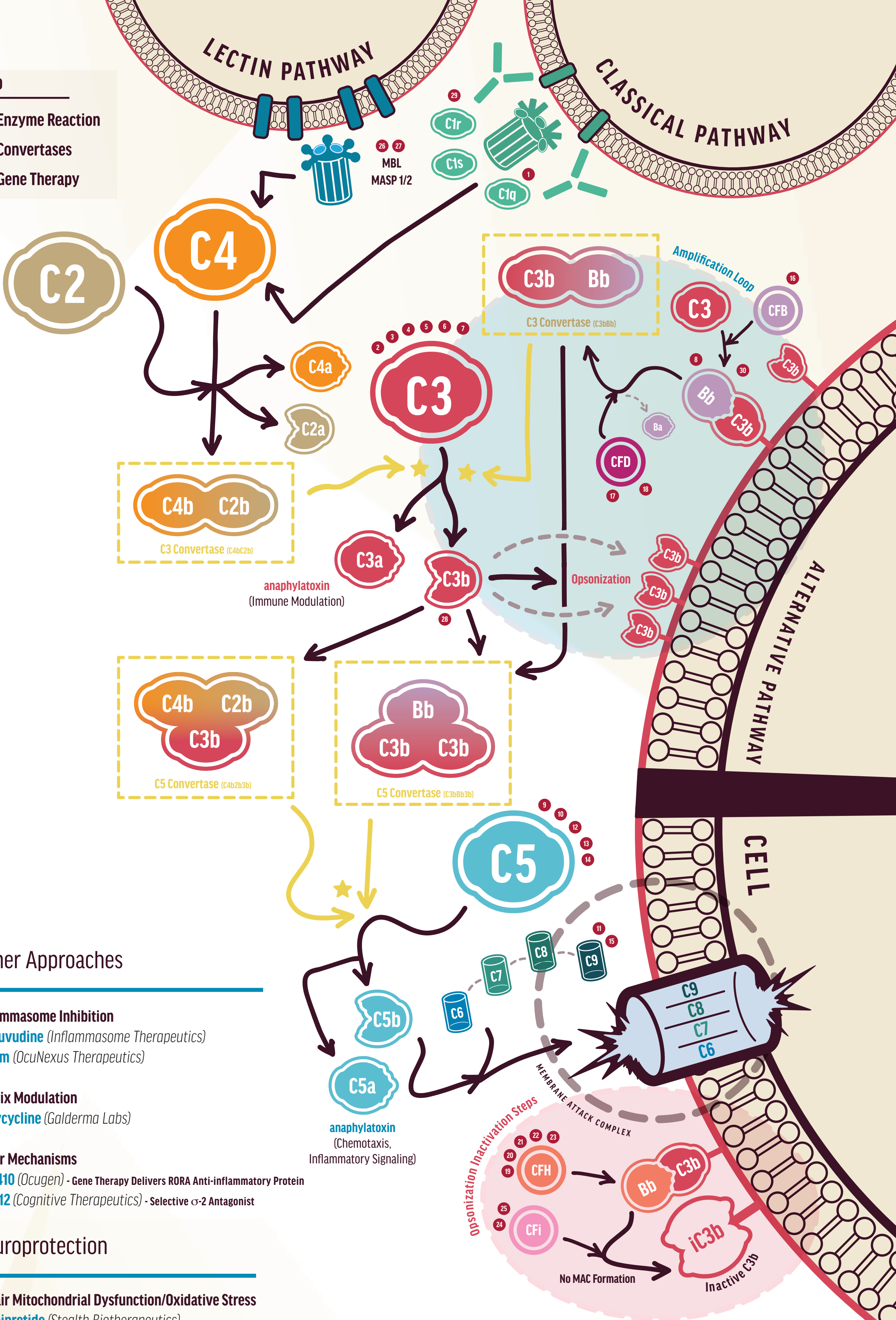
3 Stem Cells

Human Embryonic Stem Cells (hESCs)

- OpRegen** (Lineage Cell Therapeutics, Genentech/Roche)
- CPCB-RPE1 Implant** (Regenerative Patch Technologies)
- MA09-hRPE** (Astellas Pharma)
- hESC RPE Sheets** (Pfizer)
- HLS001 Cell Sheets** (Sumitomo Dainippon Pharma Co., Ltd)
- Umbilical Stem Cells (hUTCs)**
- Human Umbilical Stem Cells** (iCyte)

LEGEND

- ★ Enzyme Reaction
- Convertases
- ⌘ Gene Therapy



4 Other Approaches

Inflammasome Inhibition

- kamuvudine** (Inflammasome Therapeutics)
- Xiflam** (OcuNexus Therapeutics)

Matrix Modulation

- doxycycline** (Galderma Labs)

Other Mechanisms

- OCU410** (Ocugen) - Gene Therapy Delivers RORA Anti-inflammatory Protein
- CT1812** (Cognitive Therapeutics) - Selective α-2 Antagonist

5 Neuroprotection

Repair Mitochondrial Dysfunction/Oxidative Stress

- elamipretide** (Stealth Biotherapeutics)
- risuteganib** (Allegro Ophthalmics)
- photobiomodulation** (LumiThera)
- Oculogenex** - Gene Therapy to Reduce Oxidative Stress

6 Visual Cycle Modulation

- ALK-001** (Alkeus Pharmaceuticals)

7 HtrA1 Inhibition

- IC500** (Iveric Bio)

