Foundation Fighting Blindness

Accelerating Translation of New Treatments for IRDs: A Foundation's Perspective

July 27, 2019

FOUNDATION FIGHTING BLINDNESS

Foundation Fighting Blindness

Our Mission

The urgent mission of the Foundation Fighting Blindness is to drive the research that will provide preventions, treatments and cures for people affected by retinitis pigmentosa, age-related macular degeneration, Usher syndrome and the entire spectrum of retinal degenerative diseases.

Today, Foundation Fighting Blindness is the world's leading private funder of retinal disease research.

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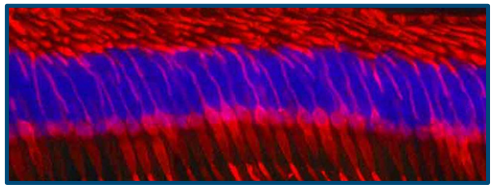
Our Beginnings

Gordon & Llura Gund

Eliot Berson, MD

Massachusetts Eye and Ear
HARVARD MEDICAL SCHOOL AFFILIATE



Founded 1974

Berman-Gund Laboratory for the Study of Retinal Degenerations

Ben & Beverly Berman

Paul Sieving, MD, PhD

National Eye Institute
NATIONAL INSTITUTES OF HEALTH

Founded 1968

Mark Humayun and Eugene de Juan, Jr.

Established in 1971

Little was known about retinal degenerative diseases

Very little research was being done

No clinical trials for potential treatments

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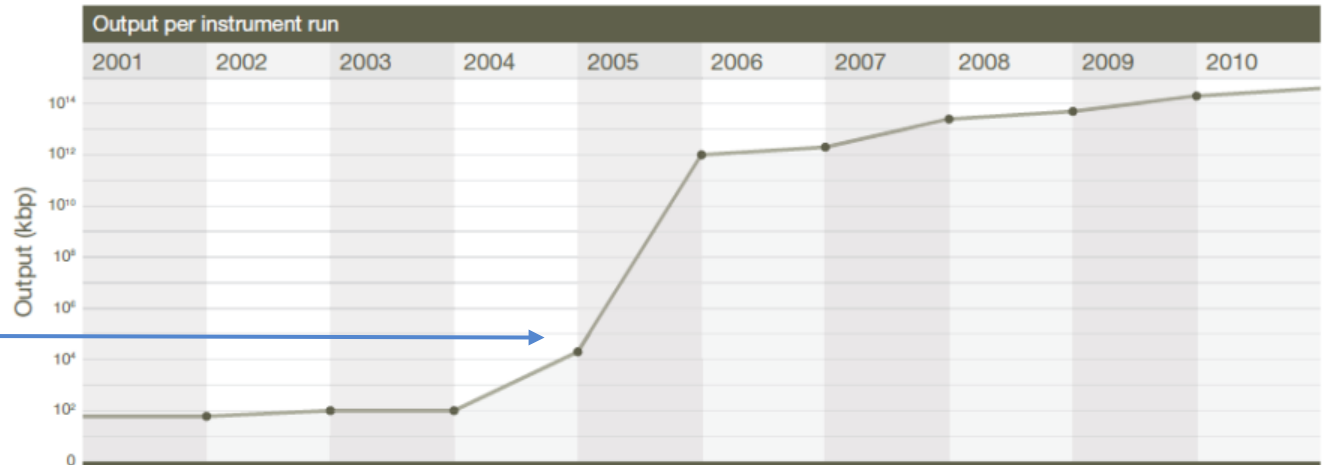
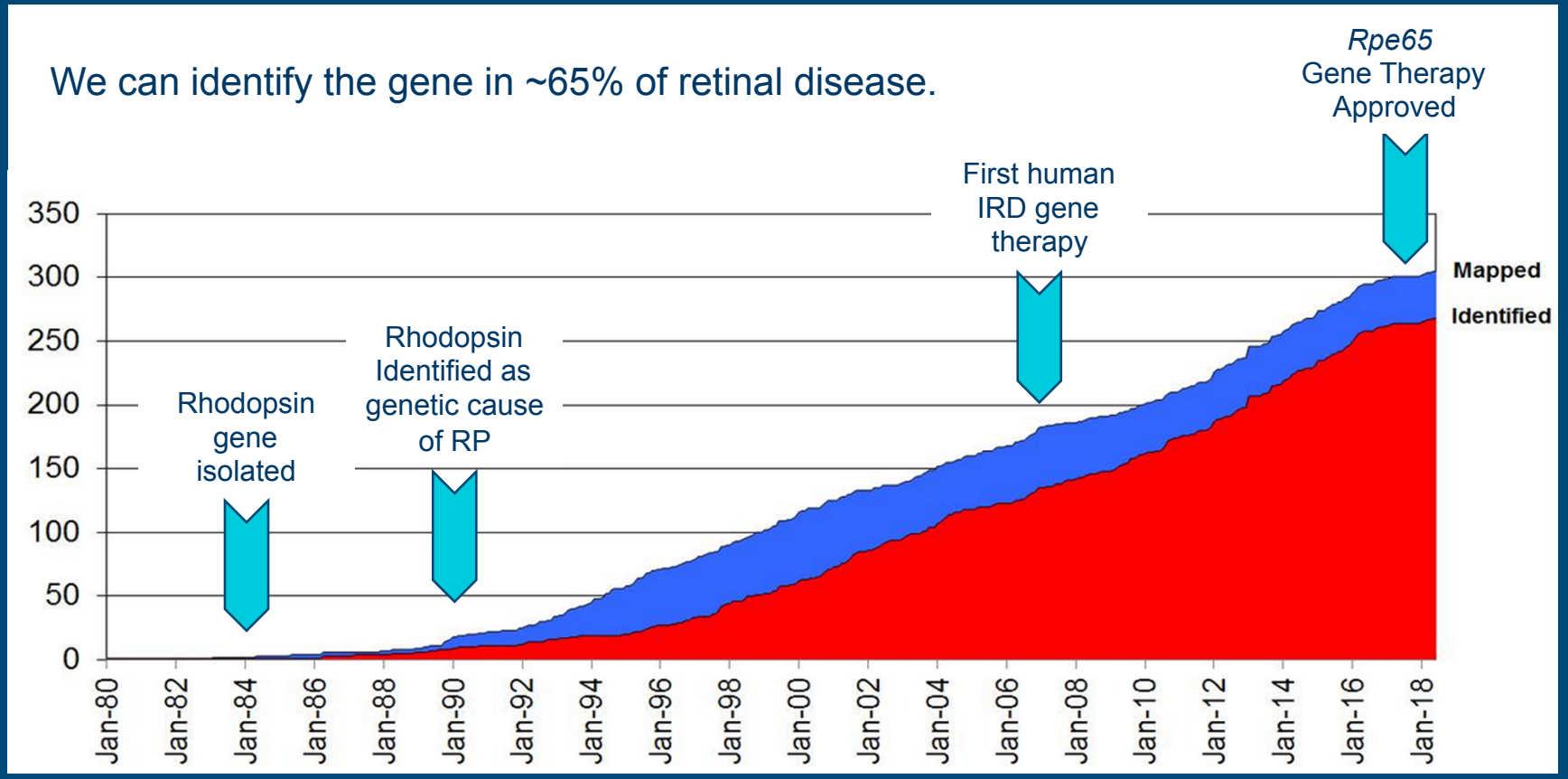
Advances in Genetic Sequencing

- Human genome project
  - Started in 1990 with a $3B budget to complete a draft sequence in 15 years
  - In 2000 a rough draft was completed and in 2003 a more complete draft published
  - Adjusted for inflation, it was a $5B project
- Riding the wave of sequencing technology advances
  - 2005 Solexa/Illumina automated sequencer
- Today, an individual can have a full genetic sequence completed in a few weeks for a couple thousand dollars
- These advances have had a tremendous impact on the IRD field



Mardis, *Nature* 2011

Understanding the Genetic Basis of Diseases



Source: RetNet, the Retinal Information Network, a service of the Laboratory for the Molecular Diagnosis of Inherited Eye Diseases

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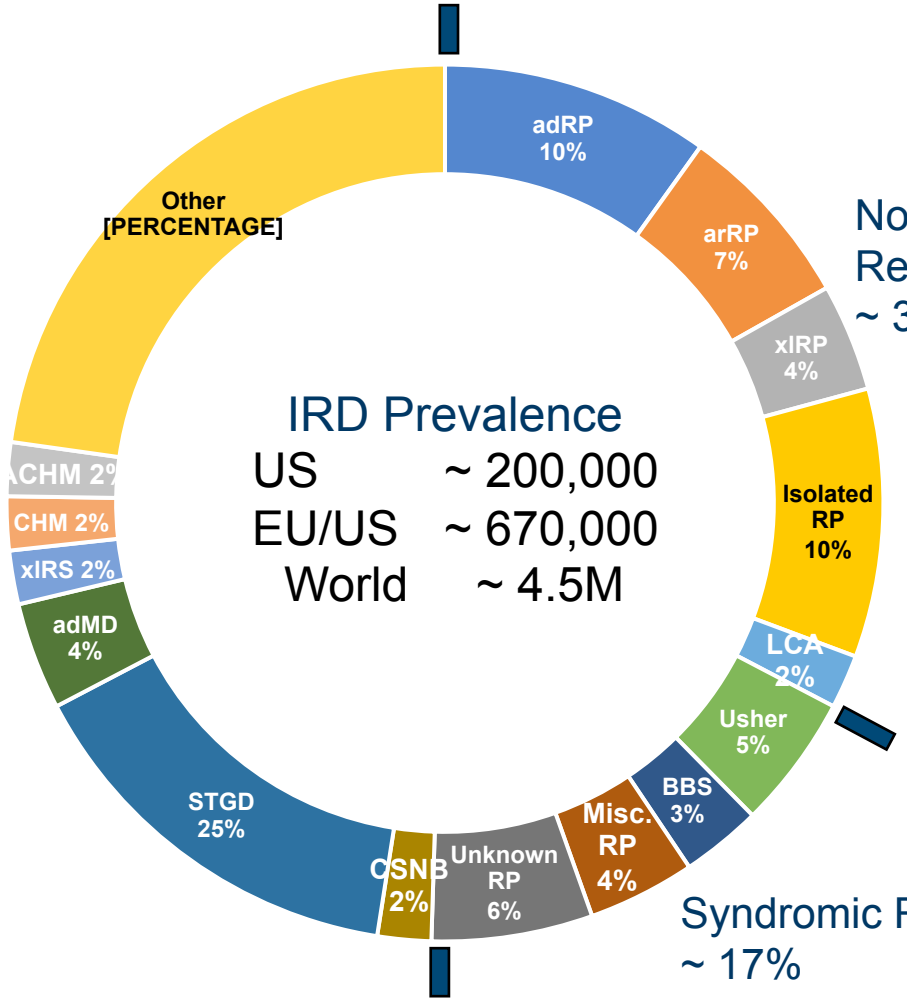
Epidemiology of Inherited Retinal Degenerations (IRDs)

IRDs as a category represent more than 200,000 patients in the U.S. alone; each condition meets the definition of orphan disease.

270+

Retinal Disease-Causing Genes/Loci Identified



Non-Syndromic Retinitis Pigmentosa (RP) ~ 33%

All Other IRDs Excluding RP ~ 50%

Syndromic RP ~ 17%

Source: RetNet, the Retinal Information Network, a service of the Laboratory for the Molecular Diagnosis of Inherited Eye Diseases

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5-Year Strategy to Further Advance Research

Awards Programs

- Career development awards
- Individual investigator awards
- Program project awards
- Translational research funding awards
- Co-funding opportunities and venture philanthropy

My Retina Tracker and Clinical Consortium

- My Retina Tracker and genetic testing
- Natural history Studies
- Develop new tools and endpoints

Awareness, Expertise and Education

- Landscapes, gap analysis and utilization of SAB expertise
- Scientific meetings and symposia of relevant research
- Public health education/ constituent support/policy advocacy

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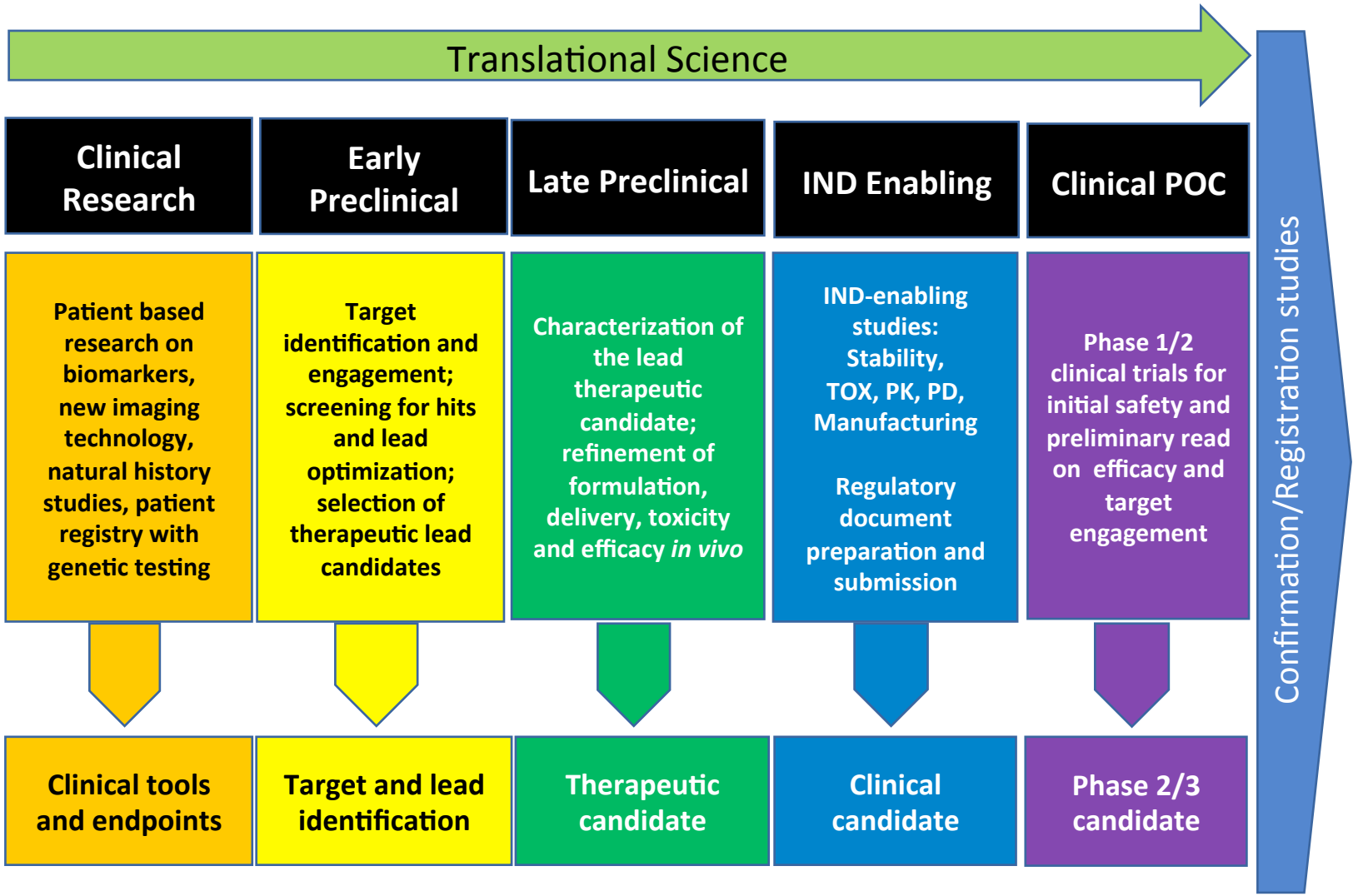
Funding Paradigm



Clinical Consortium

1	Michigan (Kellogg Eye Center)
2	OHSU (Casey Eye Institute)
3	Cincinnati Eye Institute
4	Columbia University
5	Baylor (Alkek Eye Center)
6	Emory Eye Center
7	Belgium (Ghent University)
8	Harvard Univ. (Mass. Eye and Ear Infirmary)
9	Medical College of Wisconsin
10	Moorfields Eye Hospital
11	National Eye Institute
12	Paris (Institut de la Vision)
13	Radboud University Medical Center
14	Retina Foundation of the Southwest
15	Rutgers University
16	Univ. of Penn (Scheie Eye Institute)

17	University of California San Francisco
18	Toronto (Hospital for Sick Children)
19	Germany (University of Tubingen)
20	Utah (John Moran Eye Center)
21	Vitreo Retinal Associates
22	JHU (Wilmer Eye Institute)
23	Duke Eye Center
24	Colorado Retinal Associates
25	University of Arkansas (Jones Eye Institute)
26	UNC (Kittner Eye Center)
27	Medical University of Lublin, Poland
28	Stanford University School of Medicine (Byers Eye Institute)
29	University of Miami Miller School of Medicine (Bascom Palmer Eye Institute)
30	University of Wisconsin Madison Dept of Ophthalmology & Visual Science
31	Associated Retina Consultants LTD, Phoenix
32	University of California San Diego (Jacobs Retina Center)

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Clinical Research

PROG STAR

The Natural History of the Progression of Atrophy Secondary to Stargardt disease

$6MM Foundation sponsored study of Stargardt patients over 24 months (n=460 eyes), including:

- Fundus autofluorescence, microperimetry, segmented OCT and more

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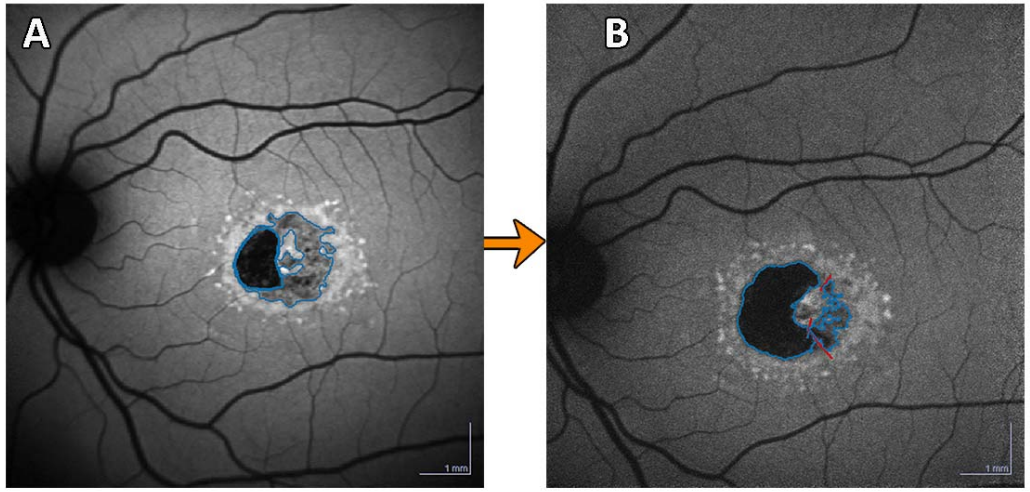
New Endpoints

PROG STAR



Fundus Autofluorescence:

- Growth of DDAF lesions (black) and total lesion size over 24 months

Segmented OCT and other endpoints also completed

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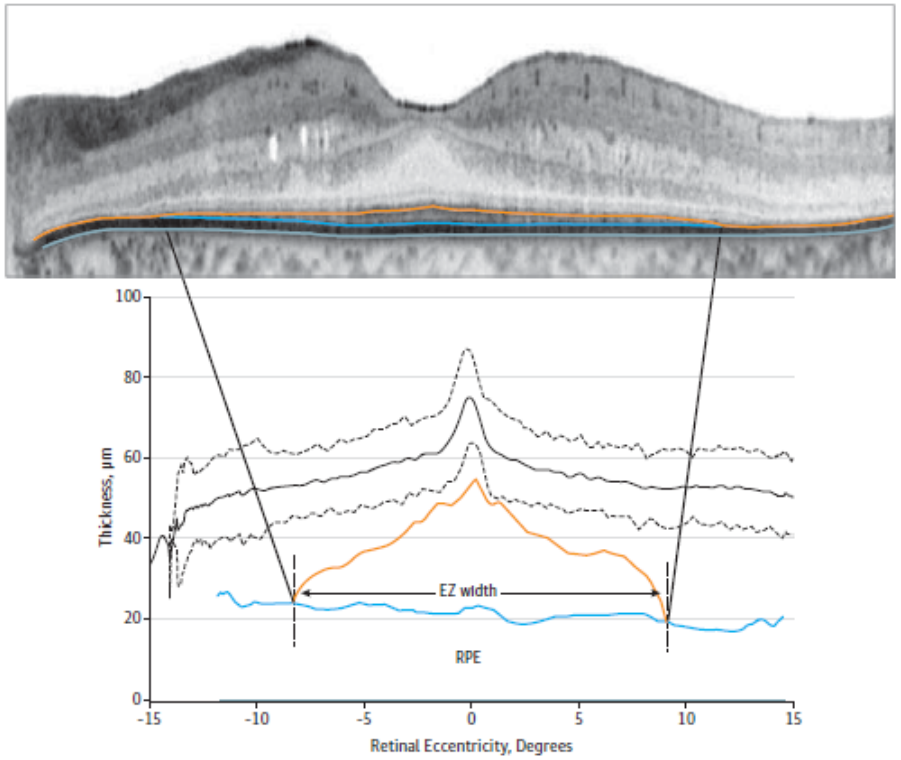
EZ Area



Article

Reliability of a Manual Procedure for Marking the EZ Endpoint Location in Patients with Retinitis Pigmentosa

Rithambara Ramachandran[1], Cindy X. Cai[1], Dongwon Lee[1], Benjamin C. Epstein[1], Kirsten G. Locke[2], David G. Birch[2,3], and Donald C. Hood[1,4]

Original Investigation

Quantification of Ellipsoid Zone Changes in Retinitis Pigmentosa Using en Face Spectral Domain–Optical Coherence Tomography

Amir H. Hariri, MD; Hong Yang Zhang, MD; Alexander Ho, BSE; Peter Francis, MD; Richard G. Weleber, MD; David G. Birch, PhD; Frederick L. Ferris III, MD; Srinivas R. Sadda, MD; for the Trial of Oral Valproic Acid for Retinitis Pigmentosa Group

Original Investigation | CLINICAL SCIENCES

Spectral-Domain Optical Coherence Tomography Measures of Outer Segment Layer Progression in Patients With X-Linked Retinitis Pigmentosa

David G. Birch, PhD; Kirsten G. Locke, BA, BS; Yuquan Wen, PhD; Kelly I. Locke, MS; Dennis R. Hoffman, PhD; Donald C. Hood, PhD

Retina

A Comparison of Progressive Loss of the Ellipsoid Zone (EZ) Band in Autosomal Dominant and X-Linked Retinitis Pigmentosa

Cindy X. Cai,[1] Kirsten G. Locke,[2] Rithambara Ramachandran,[3] David G. Birch,[2,4] and Donald C. Hood[3,5]

Research Opportunities

Report From the NEI/FDA Endpoints Workshop on Age-Related Macular Degeneration and Inherited Retinal Diseases

Karl Csaky,[1] Frederick Ferris III,[2] Emily Y. Chew,[2] Prashant Nair,[3] Janet K. Cheetham,[4] and Jacque L. Duncan[5]

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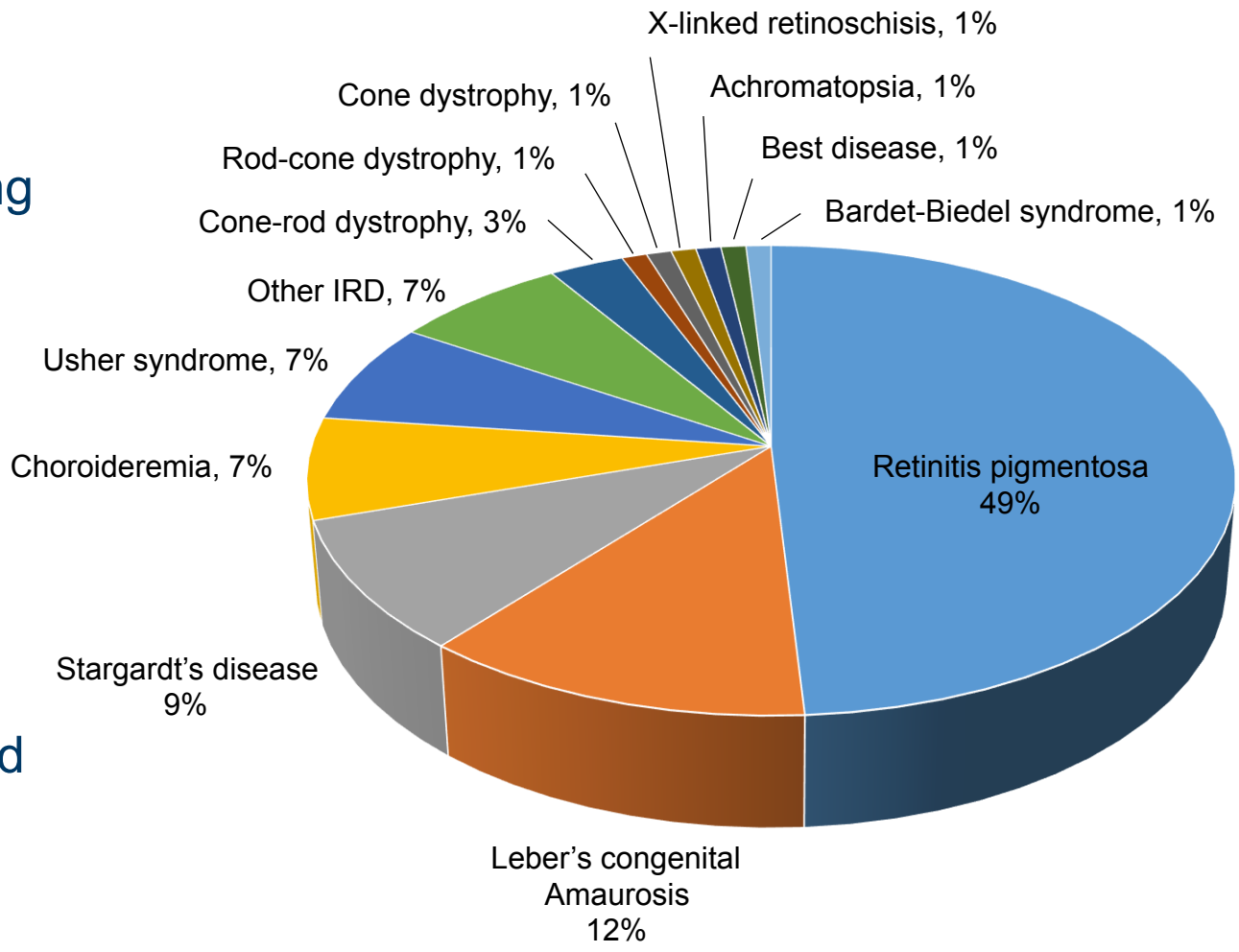
My Retina Tracker

Patient registry is international and growing with over 400 new participants per month providing rich dataset.

- Membership 23,700+
- Profiles 12,800+
- Profiles non-US 1,000+
- Genetic data 30% have been tested
- Genetic reports received to date 5,700+



Genetic Testing Study

Rapid growth in genetic testing study forces need for additional funding to continue at this pace.

- Eligibility – member, My Retina Tracker
- Enrich the value of the registry
- No cost comprehensive gene panel test
- Includes genetic counseling
- Participants to date: 5,700+
- Clinicians participating: 228+

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MY RETINA TRACKER®
Track Your Vision. **Drive the Research.**

Blueprint Genetics

Ocular Genomics Institute

InformedDNA®
Genetics, Decoded.

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LCA By The Numbers

- LCA1 (GUCY2D)
  - Boye (UF) → Sanofi, Phase 1/2
- LCA2 (RPE65)
  - Bennett (Penn) → Spark, Luxturna
  - Ali (UCL) → Meira, Phase 1/2 complete
- LCA3 (SPATA7)
- LCA4 (AIPL1)
  - Ali (UCL) → Meira, Phase 1/2
- LCA5 (Lebercilin)
  - Bennett (Penn) → Limelight, Preclinical
- LCA6 (RPGRIP1)
  - Vandenberghe (Harvard) → PTC, Preclinical
- LCA7 (CRX)
- LCA8 (CRB1)
  - Wijnholds (LUMC) → Preclinical
  - Garanto (Radboud) → Research
  - Kay (Duke) → Research
- LCA9 (NMAT1)
  - Apte (Wash U) → NMN Supplementation, Preclinical
- LCA10 (CEP290)
  - Collin (Radboud) → ProQR, Phase 3
  - Zhang (Broad) → Editas, Phase 1/2
  - Khanna (UMass) → Iveric, Preclinical
- LCA11 (IMPDH1)
- LCA12 (RD3)—Molday (UBC)
- LCA13 (RDH12)
  - Thompson (UCL) → Preclinical
  - Bennett (Penn) → Preclinical
- LCA14 (LRAT)
- LCA15 (TULP1)
- LCA16 (KCNJ13)
- LCA17 (GDF6)
- LCA18 (PRPH2)
- LCA (NPHP5/IQCB1)
  - Baehr (Utah) → Preclinical

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LCA1 (GUYCY2D)

Shannon E. Boye, Ph.D. (U Florida)

Gene Therapy Fully Restores Vision to the All-Cone *Nrl*$^{-/-}$ *Gucy2e*$^{-/-}$ Mouse Model of Leber Congenital Amaurosis-1

Sanford L. Boye,[1] James J. Peterson,[1] Shreyasi Choudhury,[1] Seok Hong Min,[1] Qing Ruan,[1] K. Tyler McCullough,[1] Zhonghong Zhang,[1] Elena V. Olshevskaya,[2] Igor V. Peshenko,[2] William W. Hauswirth,[1] Xi-Qin Ding,[3] Alexander M. Dizhoor,[2] and Shannon E. Boye[1,*]

[1]Department of Ophthalmology, College of Medicine, University of Florida, Gainesville, Florida. [2]Department of Basic Sciences Research, Salus University, Elkins Park, Pennsylvania. [3]Department of Cell Biology, College of Medicine, University of Oklahoma, Oklahoma City, Oklahoma.

When JJ was five months old he was diagnosed with LCA 1 (Leber Congenital Amaurosis). The family was happy to have a diagnosis but left with a major emotional and financial burden. They, like most families with rare diseases, had to search for answers on their own. JJ's family found help from the Foundation Fighting Blindness (FFB), where they met other families in similar positions, helping them regain hope and move forward.

SANOFI

Licensed and US IND filed

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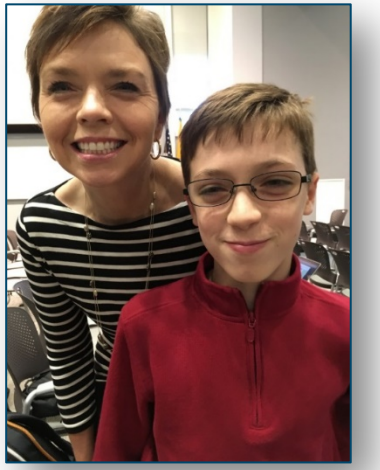
LCA2 (RPE65)

Jean Bennett, MD, Ph.D. (U Penn)

Spark THERAPEUTICS

LUXTURNA™
voretigene neparvovec-rzyl
for subretinal injection

Approved!



Ashley and Cole Carper traveled from Little Rock, AR, to tell their family's story at the FDA hearing

Robin Ali, Ph.D. (UCL)

MEIRAGTx

Phase 1/2 complete

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LCA4 (AIPL1)

Robin Ali, Ph.D. (UCL)

Gene therapy for retinitis pigmentosa and Leber congenital amaurosis caused by defects in *AIPL1*: effective rescue of mouse models of partial and complete Aipl1 deficiency using AAV2/2 and AAV2/8 vectors

Mei Hong Tan[1], Alexander J. Smith[1], Basil Pawlyk[2], Xiaoyun Xu[2], Xiaoqing Liu[2], James B. Bainbridge[1], Mark Basche[1], Jenny McIntosh[3], Hoai Viet Tran[1], Amit Nathwani[3], Tiansen Li[2,*], and Robin R. Ali[1,*]

MEIRA GTx

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LCA5 (lebercilin)

Jean Bennett, MD, Ph.D. (U Penn)

Amelioration of Neurosensory Structure and Function in Animal and Cellular Models of a Congenital Blindness

Ji Yun Song,[1] Puya Aravand,[1,10] Sergei Nikonov,[1,2,10] Lanfranco Leo,[1] Arkady Lyubarsky,[1,2] Jeannette L. Bennicelli,[1] Jieyan Pan,[1] Zhangyong Wei,[1] Ivan Shpylchak,[1] Pamela Herrera,[1] Daniel J. Bennett,[1] Nicoletta Commins,[1] Albert M. Maguire,[1] Jennifer Pham,[1] Anneke I. den Hollander,[3,4,5] Frans P.M. Cremers,[4,5] Robert K. Koenekoop,[6] Ronald Roepman,[4,7] Patsy Nishina,[8] Shangzhen Zhou,[1] Wei Pan,[2,8] Gui-shuang Ying,[2,9] Tomas S. Aleman,[1] Jimmy de Melo,[1] Ilan McNamara,[1] Junwei Sun,[1] Jason Mills,[1] and Jean Bennett[1,2]

LIMELIGHT BIO™

Licensed and in pre-IND development

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LCA6 (RPGRIP1)

Luk Vandenberghe, Ph.D. (Harvard)

Replacement Gene Therapy with a Human *RPGRIP1* Sequence Slows Photoreceptor Degeneration in a Murine Model of Leber Congenital Amaurosis

Basil S. Pawlyk,[1] Oleg V. Bulgakov,[1,3] Xiaoqing Liu,[1] Xiaoyun Xu,[1] Michael Adamian,[1] Xun Sun,[1,3] Shahrokh C. Khani,[2] Eliot L. Berson,[1] Michael A. Sandberg,[1] and Tiansen Li[1,3]

Synthetic Adeno-Associated Viral Vector Efficiently Targets Mouse and Nonhuman Primate Retina *In Vivo*

Livia S. Carvalho,[1–4,†] Ru Xiao,[1–4] Sarah J. Wassmer,[1–4] Aliete Langsdorf,[2,4] Eric Zinn,[1–4] Simon Pacouret,[1–4,6] Samiksha Shah,[1–4] Jason I. Comander,[2,4] Leo A. Kim,[3,4] Laurence Lim,[4,‡] and Luk H. Vandenberghe[1–5,*]

[1]Grousbeck Gene Therapy Center, Boston, Massachusetts; [2]Ocular Genomics Institute, Department of Ophthalmology, Harvard Medical School, Boston, Massachusetts; [3]Schepens Eye Research Institute, Boston, Massachusetts; [4]Massachusetts Eye and Ear Infirmary, Boston, Massachusetts; [5]Harvard Stem Cell Institute, Harvard University, Cambridge, Massachusetts; [6]INSERM UMR 1089, University of Nantes, Nantes University Hospital, Nantes, France.

Odylia THERAPEUTICS

PTC THERAPEUTICS

Licensed and entering development

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LCA8 (CRB1)

Jan Wijnholds, Ph.D. (Leiden)

The CRB1 Complex: Following the Trail of Crumbs to a Feasible Gene Therapy Strategy

Peter M. Quinn[1], Lucie P. Pellissier[2] and Jan Wijnholds[1,2]*

[1] Department of Ophthalmology, Leiden University Medical Center, Leiden, Netherlands, [2] Unité Physiologie de la Reproduction et des Comportements, INRA UMR85, Centre National de la Recherche Scientifique UMR 7247, Institut Français du Cheval et de l'Equitation, Université François Rabelais, Nouzilly, France, [3] Netherlands Institute for Neuroscience, Royal Netherlands Academy of Arts and Sciences, Amsterdam, Netherlands

Alex Garanto, Ph.D. (Radboud)

3389 — *A0160* The shorter the better?: Assessing novel therapeutic strategies for *CRB1*-associated retinal disease. *Alex Garanto[1,2], A. Hoogendoorn[1], J. J. Lurvink[1], R. W. Collin[1,2].* [1]Human Genetics, Radboudumc; [2]Donders Institute for Brain, Cognition and Behaviour

Jeremy Kay, Ph.D. (Duke)

1740 — *12:30* *CRB1* expresses multiple isoforms in multiple retinal cell types — reevaluating *CRB1* retinopathies. *Thomas Ray, K. Cochran, W. SPENCER, M. Cady, V. Y. Arshavsky, J. Kay.* Duke University

LCA9 (NMAT1)

Rajendra Apte, MD, Ph.D. (Wash U)

NAMPT-mediated NAD+ biosynthesis is essential for vision in mice

Jonathan B. Lin[1,2,†], **Shunsuke Kubota**[1,†], **Norimitsu Ban**[1], **Mitsukuni Yoshida**[2,3], **Andrea Santeford**[1], **Abdoulaye Sene**[1], **Rei Nakamura**[1], **Nicole Zapata**[1], **Miyuki Kubota**[1], **Kazuo Tsubota**[5], **Jun Yoshino**[4], **Shin-ichiro Imai**[3,*], and **Rajendra S. Apte**[1,3,4,*]

[1]Department of Ophthalmology & Visual Sciences, Washington University School of Medicine, St. Louis, MO, USA

[2]Neuroscience Graduate Program, Division of Biology and Biomedical Sciences, Washington University School of Medicine, St. Louis, MO, USA

[3]Department of Developmental Biology, Washington University School of Medicine, St. Louis, MO, USA

[4]Department of Medicine, Washington University School of Medicine, St. Louis, MO, USA

[5]Department of Ophthalmology, Keio University School of Medicine, Tokyo, Japan

Nicotinamide mononucleotide (NMN) supplementation--Preclinical

LCA10 (CEP290)

Rob Collin, MD, Ph.D. (Radboud)

Splice-Modulating Oligonucleotide QR-110 Restores *CEP290* mRNA and Function in Human c.2991+1655A>G LCA10 Models

Kalyan Dulla,[1,4] Monica Aguila,[2,4] Amelia Lane,[2] Katarina Jovanovic,[2] David A. Parfitt,[2] Iris Schulkens,[1] Hee Lam Chan,[1] Iris Schmidt,[1] Wouter Beumer,[1] Lars Vorthoren,[1] Rob W.J. Collin,[3] Alejandro Garanto,[3] Lonneke Duijkers,[3] Anna Brugulat-Panes,[2] Ma'ayan Semo,[2] Anthony A. Vugler,[2] Patricia Biasutto,[1] Peter Adamson,[1,2] and Michael E. Cheetham[2]

ProQR

Phase 3 started

Feng Zhang, Ph.D. (Broad)

Genome engineering using the CRISPR-Cas9 system

F Ann Ran[#1,2,3,4,5], **Patrick D Hsu**[#1,2,3,4,5], **Jason Wright**[1], **Vineeta Agarwala**[1,6,7], **David A Scott**[1,2,3,4], and **Feng Zhang**[1,2,3,4]

editas MEDICINE

Allergan

Phase 2 recruiting

Hemant Khanna, Ph.D. (U Mass)

Gene Therapy Using a *miniCEP290* Fragment Delays Photoreceptor Degeneration in a Mouse Model of Leber Congenital Amaurosis

Wei Zhang,[1] Linjing Li,[1] Qin Su,[2] Guangping Gao,[2] and Hemant Khanna[1,2,*]

[1]*Department of Ophthalmology and* [2]*Horae Gene Therapy Center, UMASS Medical School, Worcester, Massachusetts.*

iVERIC bio

Licensed--preclinical

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LCA13 (RDH12)

Debra Thompson, Ph.D. (U Michigan)

Development of a gene-therapy vector for *RDH12*-associated retinal dystrophy

Kecia L. Feathers,[1] Lin Jia,[1] N. Dayanthi Perera,[1] Adrienne Chen,[1] Feriel K. Presswalla,[1] Naheed W. Khan,[1] Abigail T. Fahim,[1] Alexander J. Smith,[3] Robin R. Ali,[1,3] and Debra A. Thompson[1,2,*]

Jean Bennett, MD, Ph.D. (U Penn)

***RDH12* Mutations Cause a Severe Retinal Degeneration With Relatively Spared Rod Function**

Tomas S. Aleman,[1,2] Katherine E. Uyhazi,[1] Leona W. Serrano,[1] Vidyullatha Vasireddy,[2] Scott J. Bowman,[1] Michael J. Ammar,[1] Denise J. Pearson,[1] Albert M. Maguire,[1,2] and Jean Bennett[1,2]

[1]Scheie Eye Institute at the Perelman Center for Advanced Medicine, Philadelphia, Pennsylvania, United States
[2]Department of Ophthalmology, Center for Advanced Ocular and Retinal Therapeutics, Perelman School of Medicine, University of Pennsylvania, Philadelphia, Pennsylvania, United States

LCA (NPHP5/IQCB1)

Wolfgang Baehr, Ph.D. (Utah)

Rescue of cone function in cone-only *Nphp5* knockout mouse model with Leber congenital amaurosis phenotype

Christin Hanke-Gogokhia,[1] Vince A. Chiodo,[2] William W. Hauswirth,[2] Jeanne M. Frederick,[1] Wolfgang Baehr[1,3,4]

[1]Department of Ophthalmology, John A. Moran Eye Center, University of Utah Health Science Center, Salt Lake City, UT; [2]Department of Ophthalmology, University of Florida, Gainesville, FL; [3]Department of Neurobiology and Anatomy, University of Utah Health Science Center, Salt Lake City, UT; [4]Department of Biology, University of Utah, Salt Lake City, UT

Elise Heon, MD (Toronto)

Specific retinal phenotype in early *IQCB1*-related disease

A Vincent[1,2,3], A AlAli[1,2], H MacDonald[1,4,5], C VandenHoven[1] and E Héon[1,2,3]

Innovation in Venture Philanthropy: RD Fund

- First-of-a-kind fund focused on IRDs
- Internal, dedicated venture philanthropy investment fund
  - Independent board of directors
- Launched with over $70M under management
  - Includes previous 3 investments made under old CRI structure
- All returns go back to support the Foundation's mission
- Seeking to make investments in IRD and select AMD assets
  - Must be ready for clinical testing in 18-24 months
- Actively raising additional funds via major gift donations
- More info at www.RetinalDegenerationFund.org

RD FUND
RetinalDegenerationFund.org

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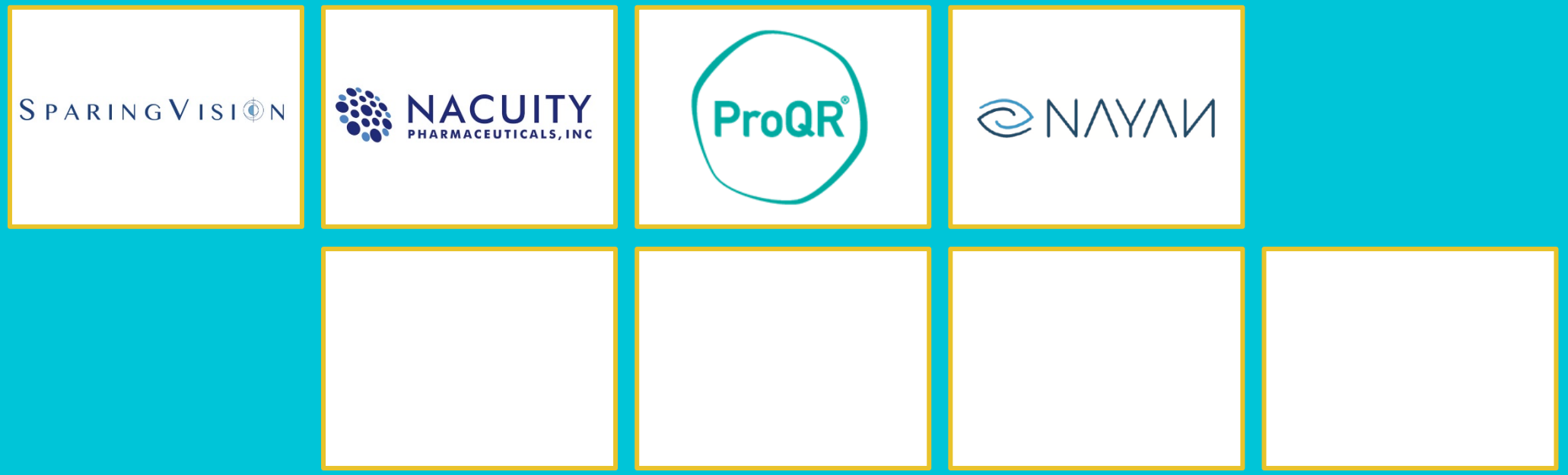
RD Fund Portfolio



Our Space is Very Active

Clinical Trial Pipeline – 36 Trials (select) in IRDs & Dry AMD

Gene Therapies	Progress
Achromatopsia (CNGB3) – AGTC	Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx	Phase 1/2
Achromatopsia (CNGA3) – AGTC	Phase 1/2
Achromatopsia (CNGA3) – Tubingen Hosp	Phase 1/2
AMD (Dry) – Gyroscope	Phase 1/2
Choroideremia (REP1) – Nightstar	Phase 3
Choroideremia (REP1) – Spark	Phase 1/2
Choroideremia (REP1) – Tubingen Hosp	Phase 2
LCA and RP (RPE65) – MeiraGTx	Phase 1/2
LCA and RP (RPE65) – Spark	FDA Approved
RP (PDE6B) – Horama	Phase 1/2
RP, Usher, others (optogenetic) – Allergan	Phase 1/2
RP, Usher, others (optogenetic) – GenSight	Phase 1/2
RP (RLBP1) – Novartis	Phase 1/2
Retinoschisis (RS1) – AGTC	Phase 1/2
Retinoschisis (RS1) – NEI	Phase 1/2
Stargardt disease (ABCA4) – Sanofi	Phase 1/2
Usher syndrome 1B (MYO7A) – Sanofi	Phase 1/2
X-linked RP (RPGR) – AGTC	Phase 1/2
X-linked RP (RPGR) – MeiraGTx	Phase 1/2
X-linked RP (RPGR) – Nightstar	Phase 3 Pen.

Cell-Based Therapies	Progress
AMD-dry (RPE) – Astellas	Phase 1/2
AMD-dry (RPE) – Cell Cure	Phase 1/2
AMD-dry (RPE on scaffold) – Regen Patch	Phase 1/2
RP, Usher (retinal progenitors) – jCyte	Phase 2b
RP, Usher (retinal progenitors) – ReNeuron	Phase 2
Stargardt (RPE) – Astellas	Phase 1/2

Molecules, Proteins, AONs, CRISPR	Progress
AMD-dry (C3 inhibitor) – Apellis	Phase 3
AMD-dry (C5 inhibitor) – Ophthotech	Phase 2
Bardet-Biedl (metformin) – Tubingen Hosp	Phase 2 Pen.
LCA (CEP290, AON) – ProQR	Phase 1/2
LCA (CEP290, CRISPR) – Editas	Phase 1/2 Pen.
Stargardt disease (emixustat) – Acucela	Phase 3
Stargardt disease (deuterated vit A) – Alkeus	Phase 2
Stargardt disease (C5 inhibitor) – Ophthotech	Phase 2
Usher syndrome 2A (AON) -- ProQR	Phase 1/2 Pen.

Visit **www.ClinicalTrials.gov** for more details and trial contact information. This document is for informational purposes only. Information is subject to change, and its accuracy cannot be guaranteed. Updated March 2019.

National Conferences

The Foundation Fighting Blindness VISIONS conference brings together the world's leading vision scientists, assistive technologies experts, and the visually impaired community.

Co-hosted with Casey Eye Institute at Oregon Health & Science University, the annual Retinal Cell and Gene Therapy Innovation Summit showcases representatives from industry and academia to discuss rapidly emerging ocular cell and gene therapies.

The Investing in Cures Summit, now an annual event, is a conference focused on IRDs and features panel discussions from experts in the overall ecosystem covering drug discovery to drug approval.

FOUNDATION FIGHTING BLINDNESS

VisionWalk

The Foundation Fighting Blindness VisionWalk program includes more than 40 walk events in cities throughout the United States.

Each walk is a fun, family-friendly 5K and an opportunity for communities to come together in support of the Foundation's mission to fund research leading to treatments for blindness caused by retinal degenerative diseases.

Local Vision Seminars

The Vision Seminar series is intended to raise public awareness of retinal degenerative diseases, provide affected families with valuable information, and urge local communities to join in the Foundation's fight to find treatments for retinal degenerative diseases.

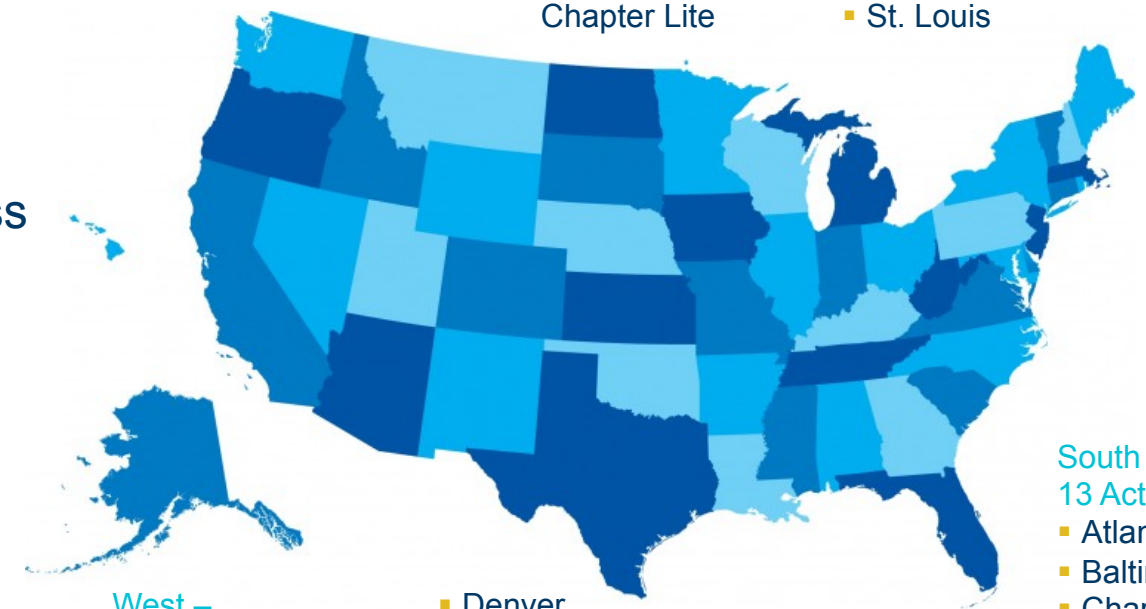
Each seminar consists of presentations given by local retinal specialists, retinal researchers, low vision doctors, and other vision resource experts, and cover topics such as:

- The latest retinal research advancements and treatments
- Updates on local clinical trials that are underway
- Genetic testing and counseling/My Retina Tracker

FOUNDATION FIGHTING BLINDNESS

Local Chapters

The Foundation Fighting Blindness has a national network of volunteer groups that raise funds, increase public awareness and provide support to their communities.



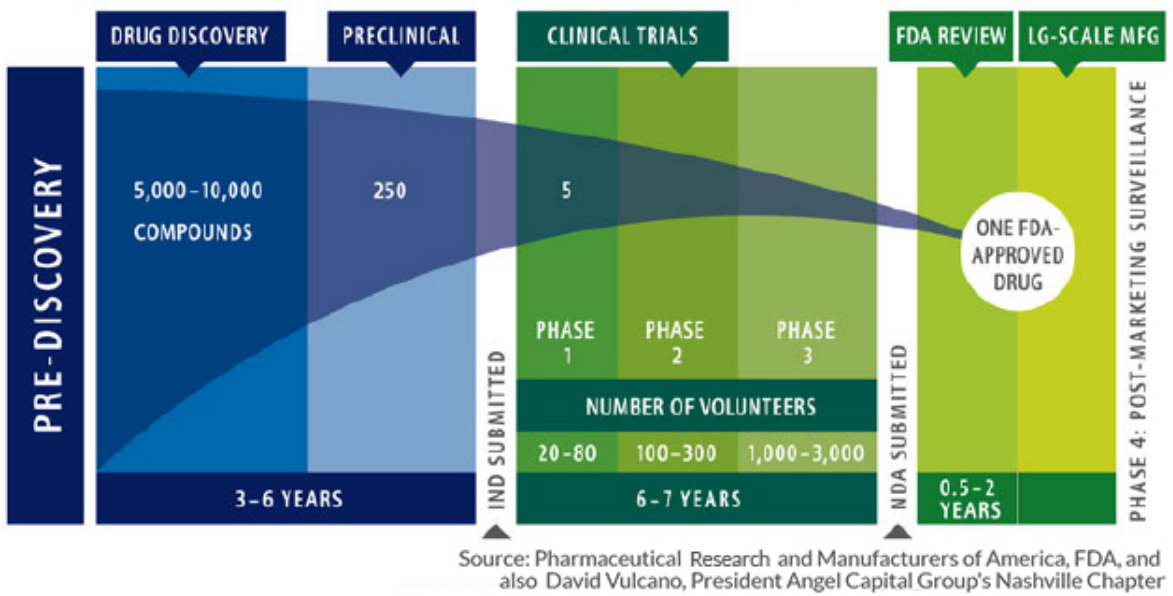
Midwest – 12 Active Chapters
- Chicago
- Cincinnati/ N. Kentucky
- Cleveland
- Columbus Chapter Lite
- Eastern Michigan
- Ft. Wayne
- Indianapolis
- Kansas City
- Milwaukee
- Minneapolis
- Pittsburgh
- St. Louis

Northeast – 6 Active Chapters
- Boston
- Long Island
- New York City
- Philadelphia
- Princeton
- Westchester/ Fairfield
- Western New York

South – 13 Active Chapters
- Atlanta
- Baltimore
- Charlotte
- Greensboro (Triad)
- Jacksonville
- Montgomery County
- Nashville
- Northern VA
- Orlando
- Raleigh-Durham
- South Carolina
- Tampa
- Virginia Beach (Hampton Roads)

West – 10 Active Chapters
- Arizona
- Bay Area
- Dallas
- Fort Worth
- Denver
- Houston
- Los Angeles
- Orange County
- San Antonio
- San Diego
- Seattle

Eye Bonds



Market Watch, Oct 28, 2018

THE WALL STREET JOURNAL.
WSJ

Karen Petrou

FINANCIAL REGULATION

The Woman Who Wants Wall Street to Fund a Cure for Blindness

Karen Petrou spent years trying to hide her blindness. Now she has come up with a plan to get private investors to bankroll a cure.

FOUNDATION FIGHTING BLINDNESS

Get Involved

Broad range of ways for *anyone* to be involved and we are open to new innovative ideas to expand our reach.

Thank You!

Ben Yerxa
CEO
byerxa@fightingblindness.org

FOUNDATION FIGHTING BLINDNESS