



# **Patient Power**

## **The Importance of Clinical Research from the Patient Perspective**

Caitríona Dunne, Communications & Advocacy Executive, Fighting Blindness

# Our Vision



Our vision is to **cure** blindness;  
to **support** people living with sight  
loss; and to **empower** patients.

# The Facts

## Number of People Affected by Sight Loss



### Worldwide

45,000,000



55,000,000 by 2020



### Ireland

224,000



272,000 by 2020

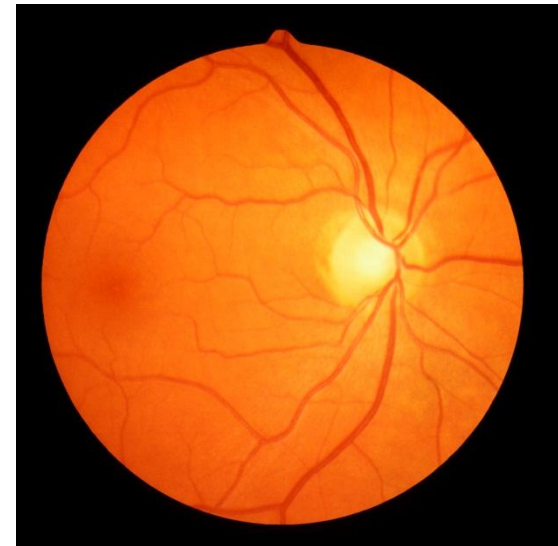
# Conditions

## Rare Genetic Conditions

- Retinitis Pigmentosa (RP)
- Choroideremia
- Leber Congenital Amaurosis (LCA)
- Usher Syndrome
- Stargardt Disease
- Juvenile X-linked Retinoschisis
- Leber Hereditary Optic Neuropathy (LHON)
- Best Disease
- Cone-Rod Dystrophies
- Achromatopsia

## More common conditions

- Age-related macular degeneration
- Diabetes related sight loss



# Where we started

- Families affected by the conditions coming together to support each other
- No hope of treatments or cures
- Inherited, genetic conditions can affect many people in one family



Limited Sight.....

# What we did

## .....Unlimited Vision



- Raised IR£150,000 in the early 1980s
- Seed funded a project at Trinity College Dublin
- First group in the world to make RP gene discovery
- To date we have invested over €15 million in more than 70 projects

# Where we are now



Global leaders in the search for cures and treatments for genetically inherited and age-related forms of vision loss

# Cure



# Pillars of Research

**Genes & Gene Therapy**

**Cell Therapy and Regenerative Medicine**

**Retinal Implant Technology**

**Novel Drug Therapy**

**Population Studies**



Aim:

- To provide genetic testing for the estimated 5,000 people in Ireland who have a genetic retinal condition
- To build a National Patient Registry for Inherited Retinal Degenerations

Project led by three ophthalmologists;

- Dr Paul Kenna, Royal Victoria Eye and Ear Hospital, Dublin
- Mr David Keegan, Mater Misericordiae University Hospital, Dublin
- Ms Giuliana Silvestri in the Royal Victoria Hospital, Belfast

Two clinical fellows:

- Dr Conor Malone, Royal Victoria Eye and Ear Hospital
- Dr Kirk Stephenson, Mater Misericordiae University Hospital

Post-doctoral fellow;

- Dr Matthew Carrigan, Trinity college Dublin

# Support

# Insight Counselling Service

- Established in 2002 to provide support for people and families affected by sight loss
- Professional counselling service
- Serves more than 400 people per year
- Expanding to become a national service



# Support Services

- Individual, couple and family therapy
- Peer support groups
- Telephone counselling service
- The Exchange Club
- Visionaries Music School
- Hospital supports

# Empower

# Nothing About Us Without Us

- A central goal
- Patient-led organisation
- Work in Ireland, Europe and on international level
- Aim to ensure that the patients' voice is heard and included in any decisions made about us
- We empower ourselves through knowledge, experience, collaboration and the collective voice to enable our common goal

# Patient Advocacy

- National Vision Strategy for Ireland
- National Plan for Rare Diseases
- Development of sustainable research infrastructures to enable the development of therapies for unmet medical needs
- Development of clinical trials infrastructure in Ireland
- Access to current and emerging therapies
- Health technology assessment



# The Patient Perspective

- Diverse patient group
- Varied levels of engagement
- Therapies at different stages of research and development
- Informed actors - Equal partners
- Natural History Studies
- Patient relevant clinical endpoints
- Patient reported outcomes

# Closing The Loop



EXPERT/PIONEERING/CONNECTED



# Thank You



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