NIHR | Clinical Research Facility

NIHR CLINICAL RESEARCH FACILITY: MOORFIELDS

Transforming vision with experimental medicine

Impact report

The world's largest ophthalmic clinical research facility

20 Years

Moorfields Eye Hospital NHS Foundation Trust

UCL Institute of Ophthalmology

A MESSAGE FROM

Professor Sobha Sivaprasad

Director, NIHR Moorfields Clinical Research Facility



On behalf of my team and the thousands of patients who make our work possible, I am pleased to present this report, marking over a decade of pioneering work at the NIHR Moorfields Clinical Research Facility. Since opening in 2012, we have grown to become the world's largest ophthalmic experimental medicine and early-phase facility, hosting some of the most important clinical firsts in eye research.

The following pages tell the story of treatments that began as laboratory ideas and became realities for patients: the world's first embryonic stem cell trial in eye disease; gene therapies now available on the NHS; a bionic eye that received FDA approval; and laser treatment that changed global glaucoma guidelines. These are not just scientific milestones. They represent sight restored, blindness prevented, and independence regained for patients who had no other options.

What makes these achievements possible is a combination of world-class science, an exceptional multidisciplinary team, and the trust of thousands of patients willing to participate in experimental medicine. I am grateful to each of them, and to all the researchers, nurses, technicians, and support staff who have made this facility what it is.

Our ambition for scientific excellence is matched by our commitment to inclusion, ensuring the benefits of research reach everyone. Patients are at the heart of everything we do, and we continue working to identify, connect and include currently under-represented groups in research.

ABOUT

The world's largest ophthalmic clinical research facility

Funded by a £5.3 million award from the National Institute for Health and Care Research, the CRF opened in 2012 as a purpose-built centre for experimental medicine and early-phase clinical trials in ophthalmology. It is located within Moorfields Eye Hospital NHS Foundation Trust, the world's oldest specialist eye hospital.

In partnership with the UCL Institute of Ophthalmology, which together with Moorfields ranked first in the world for ophthalmology research, the facility has grown through successive five-yearly NIHR funding cycles. It provides the clinical delivery platform for translational research from the NIHR Moorfields Biomedical Research Centre (BRC), and attracts commercial and investigator-led studies from partners worldwide.

The CRF supports studies spanning the full spectrum of eye disease, from the most common conditions affecting millions to rare genetic disorders affecting only a handful of families.

2012

Opened as a purpose-built experimental medicine centre

16

Specialist treatment rooms with capabilities found nowhere else in the UK

#1

Moorfields & UCL rank first in the world for ophthalmology research

2

Adaptive Optics Scanning Laser Ophthalmoscopes for single-cell *in vivo* retinal imaging

IMPACT IN NUMBERS

More than a decade of delivery

Cumulative totals since the CRF's inception in April 2012

11,075
Participants in research

13,033
Recruitments to studies

62,893
Participant study visits

68
Early-phase studies

202
Commercial studies

234
Non-commercial studies

#1
Specialist NHS Trust recruiter to research

1st in Europe
Human embryonic stem cell eye trial

+150%
Recruitment increase vs pre-pandemic

A CHRONOLOGY OF FIRSTS

Landmark moments

2012

Europe's first-in-human embryonic stem cell eye trial (for Stargardt's), the world's first for any disorder. Argus II bionic eye trial is active, with Moorfields the world's largest recruiting site.

2013

FDA approves Argus II, the first regulatory-approved treatment for blindness from retinitis pigmentosa. UK's first Adaptive Optics Scanning Laser Ophthalmoscope (AOSLO) is installed in the CRF.

2014

First systemic Phase IIa trial at a standalone eye hospital. Athena Vision gene therapy spin-out attracts $20M investment. Moorfields becomes the world-leading recruiting site globally for a Thyroid eye disease TED-RV 001 (River Vision) trial.

2015

First patient injected with a stem cell-derived retinal pigment epithelium patch, part of The London Project to Cure Blindness. MeiraGTx acquires Athena Vision to commercialise Moorfields gene therapy programmes.

2016–18

Multiple first-in-human gene therapy trials are active. London Project results published in Nature Biotechnology, with patients gaining vision. First patients globally recruited to PIONEER (optogenetic gene therapy for end-stage RP).

2019–20

LiGHT trial, published in The Lancet, changes NICE & global glaucoma guidelines. Moorfields treats the UK's first NHS Luxturna patient. NICE approves Holoclar (limbal stem cell therapy); Moorfields is one of only two UK delivery centres.

2021–25

World's first 3D-printed prosthetic eye is supplied to a patient. AIPL1gene therapy world-first for childhood blindness. First UK & EU sites for trials of belzupacap sarotalocan for choroidal melanoma and tinlarebant for Stargardt disease.

01

Rare Diseases

Inherited and rare eye diseases are collectively a leading cause of blindness in working-age adults. Many carry no approved treatment. Since 2012 the CRF has built a portfolio of first-in-human trials, natural history studies and gene therapies that has made it the global reference centre in this field.

CASE STUDY 01

FIRST UK & EU SITE

DRAGON: tinlarebant for Stargardt disease

Led by Professor Michel Michaelides

Stargardt disease is the most common inherited macular dystrophy, causing severe, irreversible vision loss in childhood or early adulthood. It currently has no approved treatment. Moorfields has developed one of the largest, most comprehensively characterised Stargardt cohorts in the world.

The pivotal Phase 2/3 DRAGON trials I and II test tinlarebant, a drug that blocks RBP4 to reduce toxic lipofuscin accumulation in the retina in adolescents with early-stage disease. Moorfields was the first site to recruit globally for DRAGON I.

A companion Phase 2/3 trial of tinlarebant in adults, led by Dr Simona Esposti, is also active at Moorfields for Dry Age-related Macular Degeneration (AMD), reflecting the CRF's unmatched access to phenotyped patients across age groups.



#1
Recruitment site globally in DRAGON I & II trials

100%
Retention in DRAGON I trial

24
Month treatment period

3
Trials — adolescent & adult

CASE STUDY 02

FIRST IN HUMAN

Luce-1: gene therapy for Usher syndrome type 1B

Led by Professor Michel Michaelides · funded by Aavantgarde Bio

Usher syndrome combines profound congenital deafness with progressive retinitis pigmentosa, leading to blindness. Type 1B, caused by mutations in the *MYO7A* gene, is among the most severe forms. Until recently, no treatment existed for the retinal component.

The Luce-1 Phase 1/2 study is the first clinical trial of AAVB-081, a dual AAV8 vector delivering a functional MYO7A gene via a single subretinal injection.

To support the trial, the CRF developed a dedicated Virtual Reality (VR) maze testing area meeting FDA specifications, and trained staff in this novel functional vision assessment. This marked a significant step towards disease-modifying treatment for a condition that robs patients of both hearing and sight.

PURPOSE-BUILT FOR THE TRIAL

A dedicated Virtual Reality maze testing area

Meeting FDA specifications for functional vision assessment

1
Single subretinal injection

AAV8
Dual vector delivery

CASE STUDY 03 FIRST IN HUMAN

RAFT: stem cells for aniridia

Led by Professor Sajjad Ahmad

Aniridia-related keratopathy causes corneal clouding and severe vision loss, with no effective long-term treatment. RAFT-OS is a synthetic scaffold seeded with the patient's own limbal stem cells, transplanted onto the damaged corneal surface.

Following pre-clinical work supported by the NIHR Moorfields BRC, the first-in-human Phase I/II trial began in the CRF in 2021. It is a direct example of the translational pipeline in action, from bench discovery at UCL to first human testing at Moorfields.



NATURAL HISTORY & EARLY PHASE

Fuchs endothelial dystrophy

Led by Professor Frank Larkin

Fuchs dystrophy is a progressive disease of the corneal endothelium and one of the leading indications for corneal transplantation in the UK. As these cells die, vision deteriorates through corneal oedema and clouding.

Research at the CRF has explored novel pharmacological approaches that could treat the disease before transplantation becomes necessary. Access to one of the largest Fuchs cohorts in Europe makes Moorfields a preferred site for early-phase trials and natural history studies.



CASE STUDY 04 | FIRST IN HUMAN | NHS APPROVED

From first trial to NHS treatment

Gene therapy for LCA2 (RPE65) · Led by Professor James Bainbridge

Moorfields and UCL were among the first institutions in the world to conduct gene therapy trials for RPE65-mediated Leber Congenital Amaurosis. Long-term results, published in the New England Journal of Medicine (2015), demonstrated sustained visual improvement over three years.

These results formed part of the evidence base for voretigene neparvovec (Luxturna), which NICE recommended for NHS funding in 2019. This programme represents the complete translational arc, from first-in-human trial at Moorfields to NHS-approved treatment. Moorfields is one of four national specialist delivery centres.

2015

NEJM publishes sustained three-year visual improvement

2019

NICE recommends Luxturna for NHS funding

Feb 2020

Moorfields treats the UK's first NHS Luxturna patient, Jake Ternent, a Moorfields research participant for 15 years

CASE STUDY 06

FIRST IN UK & EU

Belzupacap sarotalocan for choroidal melanoma

Phase 3 NAIAD trial · Led by Professor Mandeep Sagoo

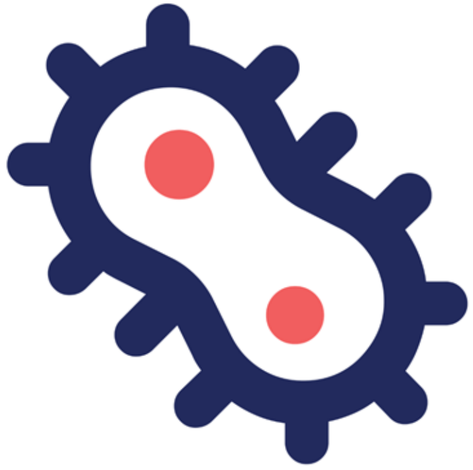
Ocular tumours are rare but potentially devastating, with many carrying a risk of metastasis as well as vision loss. The NAIAD trial evaluates **belzupacap sarotalocan (AU-011)** , a novel virus-like drug conjugate delivered intravitreally to selectively target melanoma cells in patients with indeterminate lesions or small choroidal melanoma.

Moorfields was the first site to recruit in both the UK and the European Union — part of a broader strategy to build a genuine hub-and-spoke oncology research capability, bringing complex trials into the CRF patient pathway through a collaborative model with UCLH.

A LEADING UK CENTRE

Moorfields is one of the UK's leading specialist centres for ocular oncology, with a growing portfolio of trials frequently serving as the first UK and European site for novel agents.

Professor Sagoo also serves as chief investigator for UCLH-sponsored studies, extending Moorfields' reach beyond its own patient population.



02

High Burden Conditions

Glaucoma, age-related macular degeneration, myopia and diabetic eye disease collectively affect hundreds of millions of people. The CRF's access to large, well-phenotyped populations through Moorfields' network of 30+ sites supports the running of effective trials investigating these conditions. Results have changed guidelines and saved the NHS tens of millions of pounds.

CASE STUDY 07

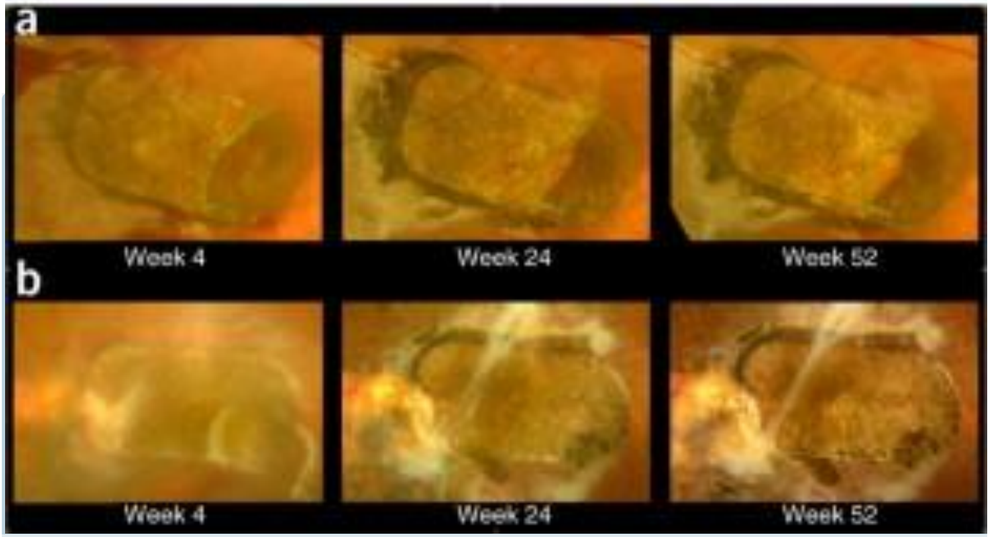
WORLD FIRST

The London Project to Cure Blindness

Stem cell patch for AMD · Led by Professor Lyndon da Cruz

In 2015, the CRF hosted the first treatment of a patient with a human embryonic stem cell-derived retinal pigment epithelium (RPE) patch. Developed in a collaboration between Moorfields, UCL and Pfizer, the patch was delivered by surgeons delivered the patch via a purpose-designed microsurgical tool into the subretinal space of patients with severe wet AMD.

All imaging, multidisciplinary assessments and follow-up visits were conducted in the CRF. The trial established proof-of-concept for retinal regenerative medicine and remains one of the most significant demonstrations of stem cell therapy in ophthalmology.



Photographs of the transplanted patch in patients 1 and 2 patch over 52 weeks. From https://doi.org/10.1038/nbt.4114

+29

Letters gained — patient one

+21

Letters gained — patient two

12-month results published in *Nature Biotechnology* (2018), with no adverse immune response requiring sustained immunosuppression.

PIPELINE OF FIRST-IN-HUMAN TRIALS

Geographic atrophy

Geographic atrophy is the advanced dry form of AMD, causing progressive loss of central vision, for which no treatment existed until very recently.

The CRF has hosted multiple first-in-human and early-phase trials. For the HORIZON Phase II trial for Geographic Atrophy (GA), the CRF recruited patients for a one-time, adeno-associated virus (AAV)-based gene therapy delivered via subretinal injection. The FOCUS study was a Phase I/II first-in-human, multi-centre study, also evaluating subretinal gene therapy involving a single injection.

This pipeline positions Moorfields patients to benefit from emerging therapies years before they reach routine clinical use.

FIRST IN HUMAN

Diabetic retinopathy

Diabetic retinopathy is the fifth commonest cause of global blindness, and diabetic macular ischaemia is an untreatable cause of sight loss in people with diabetes.

Using the CRF's retinal imaging facilities, Professor Sobha Sivaprasad conducted natural history studies (INSPIRED and DMI) to develop and validate endpoints for retinal non-perfusion.

The CRF was one of few sites worldwide to host the first-in-human HORNBILL trial, evaluating an experimental eye injection treatment for patients with moderate to severe non-proliferative diabetic retinopathy (NPDR). The results of the study supported further investigation, and the intervention has now progressed to the Phase 2 CRIMSON trial, with the CRF again serving as a site.

CASE STUDY 08 | NICE GUIDELINES CHANGED | INTERNATIONAL GUIDELINES UPDATED

LiGHT: laser as first-line glaucoma therapy

Led by Professors Gus Gazzard, Ted Garway-Heath & Gary Rubin

For decades the standard first-line glaucoma treatment was lifelong eye drops. This has been effective in principle but is compromised in practice by poor adherence, side effects and cost. The LiGHT trial, with all Moorfields patients assessed in the CRF, compared selective laser trabeculoplasty (SLT) to drops in 718 patients across six NHS trusts.

Published in The Lancet in 2019, the trial showed SLT controlled glaucoma drop-free in 75% of patients for three years, with fewer adverse events and less surgery. NICE, the World Glaucoma Association and the European Glaucoma Society updated their guidelines accordingly. Follow-on studies REVAMP and NAMinG continue to advance laser-first and neuroprotective approaches in the CRF.

75%

Controlled drop-free for 3 years

718

Patients across six NHS trusts

£250M

Estimated NHS saving from universal SLT

CASE STUDY 09

FIRST LARGE-SCALE UK TRIAL

CHAMP-UK: atropine drops for childhood myopia

A simple, low-cost intervention with NHS-wide potential

Myopia is now recognised as a global public health emergency. Half the world's population is projected to be myopic by 2050, and higher levels significantly increase the risk of retinal detachment, macular degeneration, glaucoma and cataracts. Intervening in childhood offers the best opportunity to slow progression.

CHAMP-UK is a multicentre, placebo-controlled randomised trial evaluating low-dose atropine (0.01%) eye drops in UK children. While atropine has shown strong evidence in Asian populations, no large-scale UK study had been conducted. CHAMP-UK provides the evidence base for an intervention that could be implemented across the NHS.

320%

of recruitment target achieved at the Moorfields CRF site

Demonstrating the CRF's recruitment capability in paediatric studies and its integration with Moorfields' large paediatric ophthalmology service.

03

Inclusive Research & Patient Participation

Research only delivers its full promise when it reflects the communities it serves. The CRF has invested in both the infrastructure and the culture needed to make world-class eye research accessible to everyone — particularly communities historically under-represented in clinical trials.

ROAM & COMMUNITY OUTREACH

Widening who takes part in research

Launched in 2020 under the leadership of Professor Roxanne Crosby-Nwaobi, the UK's first Black Ophthalmic Nursing Professor, **ROAM** (Research Opportunities at Moorfields) is a co-designed platform that lets patients, carers and healthy volunteers register their interest in research. By 2025 it had grown to 3,756 members.

Multilingual community outreach, including radio broadcasts in English, Hindi and Gujarati reaching over 42 million listeners, has extended awareness of research participation. Co-produced materials in seven languages and targeted engagement at Moorfields' east London satellite sites have widened reach further.

3,756
ROAM members by 2025

42M+
Radio outreach listeners

7
Languages of co-produced materials

IMPROVING DIVERSITY IN RESEARCH

Through system changes, staff training and cultural engagement led by a dedicated EDI Research Associate, the share of participants with ethnicity recorded as **unknown** fell sharply:



43%
Unknown — 2023

→

11%
Unknown — 2024–25

THE PEOPLE BEHIND THE WORK

Embedding patients at the heart of research



EDI RESEARCH

More accurate ethnicity records

Through the work of the CRF's first EDI Research Associate, Christina Adams, the recording of ethnicity among clinical trial participants improved from 43% unknown to 11% unknown. Christina has also introduced anti-racism and cultural inclusion training for all R&D staff. Her research on barriers to equitable participation in ophthalmic research was accepted for presentation at EURETINA 2025.



PAEDIATRIC RESEARCH

Including young patients

Paediatric nurse Jacqueline Miller leads Eye-YPAG, the Moorfields Young Persons Advisory Group, comprising 20 members aged 8–25. They have co-designed protocols, developed accessibility guidance adopted at conferences, and recruited young people to NHS volunteering roles. Jacqueline’s work on improving paediatric patient experience in clinical trials is extending this initiative across the UK.



PATIENT RESEARCH AMBASSADOR

Empowering participants

The Patient Research Ambassador initiative was intended to involve people with lived experience of participating in clinical trials, to bridge the research team and the patient community, The ambassador explains what participation involves and helps to normalise taking part in research, reflecting the CRF's commitment to making patient experience central to how research is designed and delivered.

NIHR | Clinical Research Facility

Preserving sight, driving equity.

The NIHR Moorfields Clinical Research Facility has been continuously funded by the National Institute for Health and Care Research since 2012, working in partnership with the NIHR Moorfields Biomedical Research Centre and NIHR CRFs across the UCL partnership.

Moorfields Eye Hospital NHS Foundation Trust is the oldest and largest specialist eye hospital in the world. Its partnership with the UCL Institute of Ophthalmology, ranked first globally for ophthalmology research, underpins its position as the world's leading centre for ophthalmic translational research.

ACKNOWLEDGEMENTS

This report is dedicated to the thousands of patients who participated in clinical trials at Moorfields CRF between 2012 and 2025. Their courage and generosity have made every discovery in these pages possible.

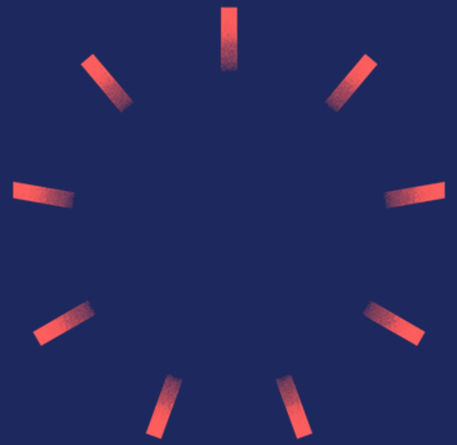
We thank Professor Frank Larkin, founding CRF Director, and all the staff, researchers, industry partners and charities who have contributed to building this facility.

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20 Years

Moorfields Eye Hospital NHS Foundation Trust

UCL Institute of Ophthalmology