ReNeuron

2021 PRELIMINARY RESULTS PRESENTATION

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RENEURON: HIGHLIGHTS





Leading clinical stage cell therapy company with presence in the UK and US

Proprietary allogeneic retinal and neural stem cell therapy platforms

Lead programme an Orphan Drug treatment with Fast Track Designation targeting retinitis pigmentosa (RP) – positive early Phase 2a clinical data with study ongoing

Planning to commence pivotal RP clinical trial in H2 2022, with data targeted for 2024, ahead of market approval application

Proprietary exosome programme – collaborations ongoing with pharma & biotech, with further collaborations anticipated

Well-funded following recent £17.5m capital raise. Extended RP Phase 2a clinical data readouts and exosome pre-clinical proof-of-concept data expected Q4 2021



PROPRIETARY PLATFORM TECHNOLOGIES



hRPC

Human Retinal Progenitor Stem Cells with sub-retinal delivery enabling engraftment

Cryopreserved formulation allows global shipand-store

Positive early Phase 2a data in ongoing retinitis pigmentosa study

Partnered with Fosun Pharma for China



Exosome Platform

High-yielding neural stem cell derived exosomes

Proven ability to load exosomes with siRNA, miRNA and proteins

Favourable distribution of exosomes across the Blood Brain Barrier

Potential as drug load/delivery vehicle and as a therapeutic. Pharma collaborations ongoing



iPSC Platform

CTX-based induced pluripotent stem cell platform

Neural stem cells
engineered into other
forms of stem cells while
preserving the
immortalisation

Potential to create allogeneic CAR-T cell therapies and pancreatic islet cells



CTX Cells

Immortalised neural progenitor stem cell line

Positive clinical data in stroke disability. Potential in Huntington's disease, TBI and other indications

Out-licensing strategy
Partnered with Fosun
Pharma for China



INTERNAL RESEARCH AND DEVELOPMENT PIPELINE

Programme	Indication	Pre-clinical	Phase 1	Phase 2	Next Milestones
Human Retinal Progenitor Cells	Retinitis Pigmentosa				 Further data read-outs from expanded Phase 2a study expected Q4 2021 Pivotal trial to commence in H2 2022, subject to Phase 2a data
Exosome platform	Neurodegeneration, Oncology, Vaccines (e.g. COVID-19)				 Additional proof of concept data from current research collaborations expected in 2021
iPSC platform	Oncology, Diabetes				 Validation of technology and publication of pre-clinical proof-of-concept data
CTX cell line	Stroke Disability				 Currently partnered in China with FOSUN 复星 Open for partnerships outside China





PROGRAMME HIGHLIGHTS





- Efficacy observed over 12 months in phase 2a study
- Study expanded with additional nine subjects and protocol changes implemented to build on the efficacy observed
- Study has reopened following a temporary suspension
- Three-month data from extension segment of Phase 2a study to be available in Q4 2021



- Further collaboration agreements signed with major pharmaceutical/biotechnology companies and leading academic institutions exploring multiple methods of loading exosomes
- Positive early pre-clinical data has shown efficient loading of nucleic acid payloads and functional payload delivery
- Exosome pre-clinical proof-of-concept data from current research collaborations is expected during Q4 2021
- New immortalised, licensable cell lines have been generated from the Company's iPSC platform as potential therapeutic agents for cancer immunotherapy and type 1 diabetes

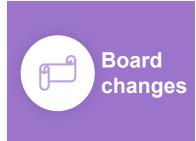


OTHER HIGHLIGHTS





- Strategic decision in June 2020 to progress stroke disability programme through regional partnerships
 - Fosun Pharma to develop and commercialise CTX programme in China under the exclusive out-licence agreement signed in April 2019
 - CTX cell therapy candidate available for licensing in stroke disability outside China and in all territories in other potential indications



- Non-executive Board membership reconfigured in September 2020 and CFO announced his retirement from the Board in March 2021
- On 1 July 2021, Iain Ross appointed as Non-Executive Chairman
- Recruitment of new Chief Financial Officer ongoing



- Successful fundraise in December 2020, raising approximately £17.5 million
- Reduced costs incurred in the period of £13.2 million (2020: £20.6 million)
- Cash, cash equivalents and bank deposits at 31 March 2021 of £22.2 million (2020: £12.6 million), providing at least a 12-month runway



PRELIMINARY RESULTS for the year ended 31 March 2021

Highlights

(£'m)	Year ended 31 March 2021	Year ended 31 March 2020
Revenues and other income	0.3	6.1
Research and development costs	(9.5)	(16.3)
General and administrative costs	(3.7)	(4.2)
Operating loss	(12.9)	(14.4)
Net finance (expense)/income	(0.5)	0.6
Taxation	2.1	2.4
Loss for the year	(11.3)	(11.4)
Net increase/(decrease) in cash and deposits	9.6	(13.8)
Cash and deposits at start of period	12.6	26.4
Cash and deposits at period end	22.2	12.6





RETINITIS PIGMENTOSA: AN UNMET NEED



RP is an inherited, degenerative eye disease^{1,2,3}

• Incidence of 1:4,000 in U.S. and worldwide



>100 genes identified containing mutations leading to RP⁴



Treatment available only for patients with a single gene defect (RPE65)

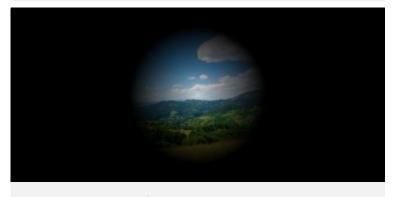


Patients with all other types of RP (c98% of patients⁵) have declining vision eventually leading to severe visual disability in most

Therapeutic benefit of hRPC approach not dependent on genetic cause



Normal View



View with Retinitis Pigmentosa



¹ Hamel (2006) Orphanet J Rare Disease 1, 40;

² https://nei.nih.gov/health/pigmentosa/pigmentosa facts;

³ NORD

⁴ https://www.genome.gov/13514348/learning-about-retinitis-pigmentosa/

⁵ www.nice.org.uk/guidance/hst11/chapter/2-The-condition

HUMAN RETINAL PROGENITOR CELLS (hRPC)





hRPC: allogeneic cell-based therapeutic approach to retinal disease



Proprietary manufacturing process and controls allow for stable, high quality and high quantity GMP production



High commercial potential

hRPCs differentiate into functional photoreceptors and integrate into retinal layers in pre-clinical models; integration may also enable durable trophic support

Broad potential across a range of eye diseases, initially targeting inherited retinal degenerative diseases

Orphan Drug Designation in EU and US in RP and FDA Fast Track Designation

Collaborations with Schepens Eye Research Institute (Harvard) and University College London

Proprietary technology enabled development of GMP manufacturing process

Cryopreserved formulation provides ninemonth shelf life and enables local treatment worldwide RP is a large orphan market.

Attractive pricing precedent set by Luxturna

Mechanism of action independent of genetic cause

Commercially viable formulation



CLINICAL DEVELOPMENT

Phases 1 and 2a

Phase 1

Single ascending dose in subjects with established RP

- Subjects with very poor visual potential
- Four cohorts, three subjects each (n=12)
- Formulation changed from fresh to cryopreserved cells

Established safety in cryopreserved formulation

Phase 2a

10 subjects with established RP

- Patients with better visual potential
- 1m cell dose

Primary endpoint

Safety

Secondary measures

Visual acuity, visual field, retinal sensitivity and retinal structure

Established efficacy signal, continued safety

Clinical Sites

Massachusetts Eye & Ear Infirmary, Boston

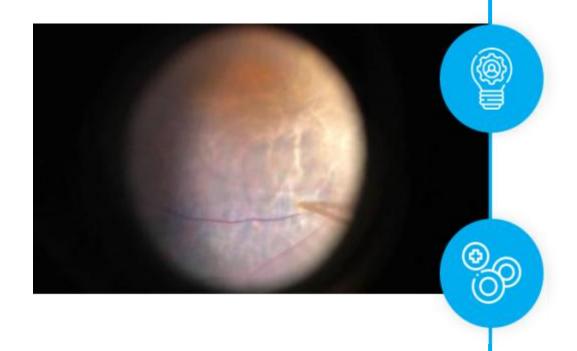
Retinal Research Institute, Phoenix



SURGICAL TECHNIQUE

Sub-retinal Injection





Well established technique used commercially with Luxturna®

Allows correct anatomic placement of cells for integration into the retina

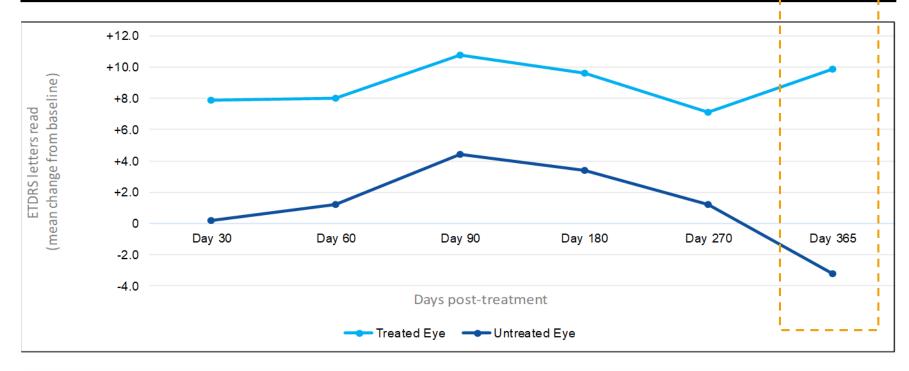
- Can serve as a depot for prolonged production of trophic factors
- Can allow for differentiation into photoreceptors with proper connections to other cells needed for vision



PHASE 2a EFFICACY RESULTS

Mean changes in ETDRS letters read (treated eye vs untreated eye)

	Day 30 (n=9)	Day 60 (n=9)	Day 90 (n=9)	Day 180 (n=9)	Day 270 (n=8)	Day 365 (n=7)
Treated Eye	+7.9	+8.0	+10.8	+9.6	+7.1	+9.9
Untreated Eye	+0.2	+1.2	+4.4	+3.4	+1.2	-3.2
Difference	+7.7	+6.8	+6.4	+6.2	+5.9	+13.1



Additional Notes:

Excluding 1 patient (6003) with surgery-related vision loss

Some patient visits have not completed due to Covid-19

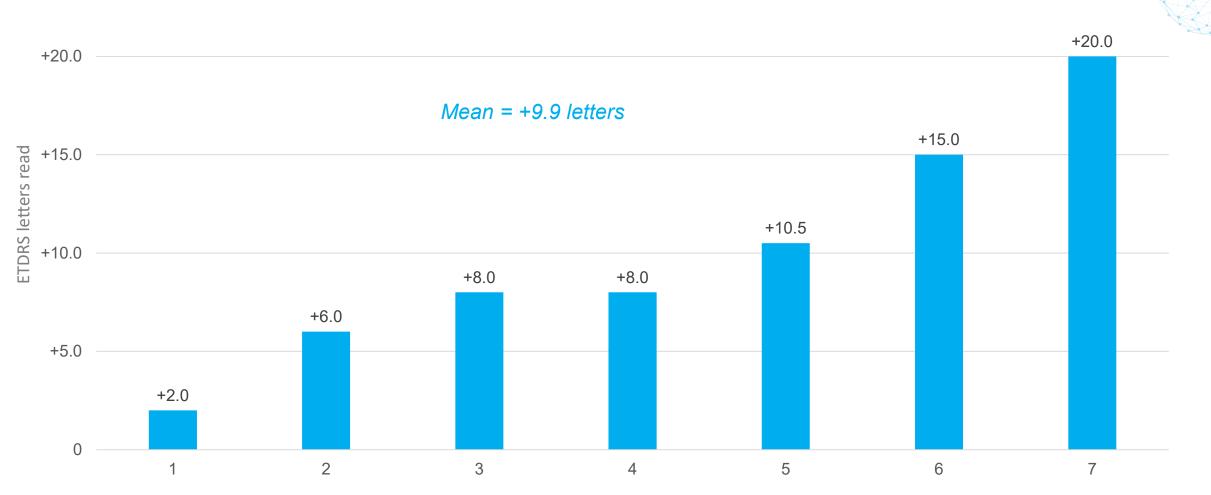


PHASE 2a EFFICACY RESULTS

INDIVIDUAL PATIENT IMPROVEMENTS AT 12 MONTHS

ETDRS change in treated eye from baseline 12 months post-treatment (n=7)

+25.0





CLINICAL DEVELOPMENT: PHASE 2a EXTENSION

Modifications to build on initial efficacy signal



9 additional subjects with established RP

- Dose escalation: from 1m to 2m cells
- Require ability to perform micro-perimetry should allow retinal sensitivity to be an indicator of efficacy
- Additional baseline VA's to ensure patient reliability
- Modified surgical technique to target bleb placement: injection sites chosen to avoid areas of viable retina

Primary endpoint

Safety

Secondary measures

 Visual acuity, micro-perimetry, visual field, retinal sensitivity and retinal structure

Additional Sites Added

Oxford Eye Hospital, Oxford, UK (Prof Robert MacLaren)

Casey Eye Institute, Oregon, US (Dr Mark Pennesi)

Institut de la Màcula, Barcelona, Spain (Dr Jordi Monés)



RETINAL PLATFORM NEXT STEPS





Recruit remaining patients in high dose expansion study

- Enhancements in patient selection, dose, surgical technique and efficacy assessments
- First cohort complete January 2021
- File for regulatory approvals to reopen for enrolment in UK and Spain

Assess other indications alongside RP (e.g. Cone Rod Dystrophy)



Further efficacy data to be presented at retinal conferences later this year

Partnering strategy to be based on full Phase 2a data



A single further clinical trial is planned before filing for marketing authorisation

- Randomised, not placebo controlled
- Three patient groups (high dose, low dose and observational cohort)



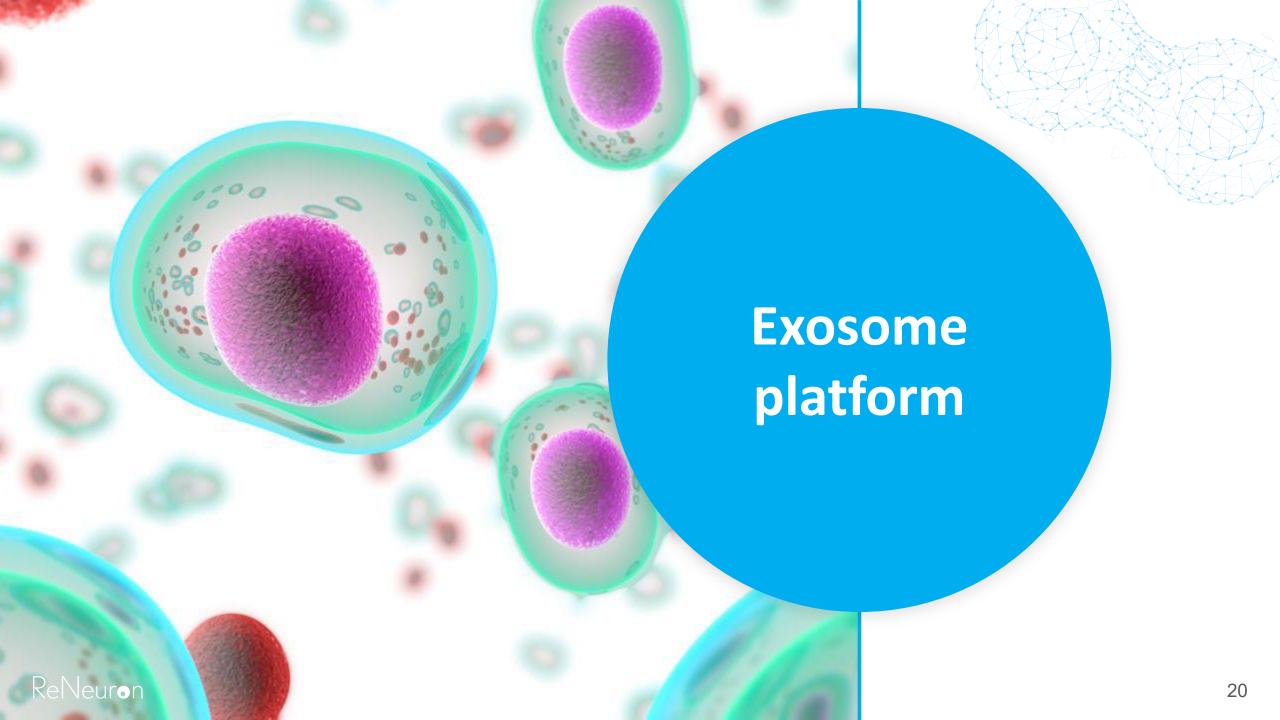
RETINITIS PIGMENTOSA: CLINICAL THERAPY LANDSCAPE

Company	Technology	Stage	Comment
ReNeuron (AIM, market cap: £63m*)	Cell therapy	Phase 1/2a	Cryopreserved formulation
jCyte Inc (US, private)	Cell therapy	Phase 2b	Not cryopreserved at drug product level
Spark Therapeutics (acquired by Roche in 2019 for \$4.3bn)	Gene therapy	Approved and marketed, Luxturna for RPE65	Addresses only about 2%** of RP patients
Nightstar Therapeutics (acquired by Biogen in 2019 for \$800 million)	Gene therapy	Phase 2/3	UK company co-founded by Prof Robert MacLaren
MeiraGTx (Nasdaq, market cap \$712m*)	Gene therapy	Phase 1/2	-
ProQR therapeutics (Nasdaq, market cap \$424m*)	RNA therapy	Phase 1/2 & Phase 2/3	-
AGTC (Nasdaq, market cap \$176m*)	Gene therapy	Phase 1/2	-



[•] Market capitalisation as at 05 July 2021

^{**} www.nice.org.uk/guidance/hst11/chapter/2-The-condition



EXOSOMES: BIOLOGICAL NANOPARTICLES



Nano-scale vesicles released by most cell types as a means of intercellular communication



Naturally occurring liposomal delivery system

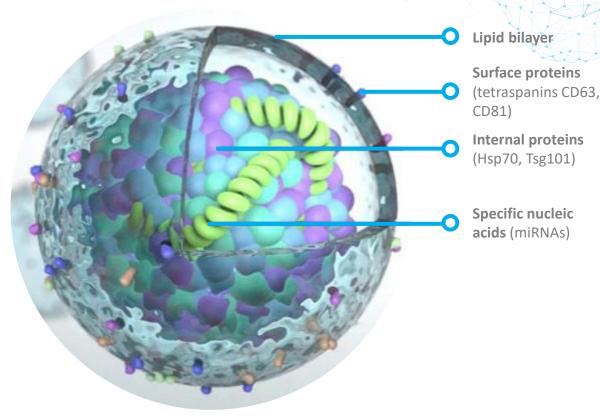


Contain and transport bio-active lipids, proteins and nucleic acids



Potential as a drug delivery vehicle and as a therapeutic

- · Current focus is on drug delivery
- · Research collaborations with pharma/biotech companies ongoing



Increasing industry interest in and commercial value of exosome deals



ADVANTAGES OF RENEURON'S EXOSOME TECHNOLOGY



Favourable distribution across the blood brain barrier



Proven ability to load miRNA and proteins



Stable, consistent, high-yield, clinical-grade product



Fully qualified xeno-free, optimised, scalable GMP process



Established analytics



Modifiable to carry siRNA/mRNA, CRISPR/Cas9 proteins, small-molecule inhibitors



Engineered to target particular tissues



PROOF OF CONCEPT DATA EXPECTED IN 2021



hNSC-Exosome Platform (for delivery across the blood brain barrier)



Significant research collaborations ongoing

- Undisclosed industry-leading companies
- Focused on delivery of siRNA and mRNA
- Goal to deliver in-vivo proof of concept data
- Trials financed by partners

Further research collaborations planned, focused on delivery of other novel therapeutics including antibodies

ReNeuron owns equal co-development rights to new therapeutic modalities





VALUE REALISATION

Recent deals in cell therapy for retinitis pigmentosa and exosomes research

Santen deal based on Phase 2 data in RP*



- jCyte Inc signed an ex-US licensing deal for its jCell product in May 2020 with Santen Pharmaceutical
- ¡Cell is a Phase 2b retinal progenitor cell suspension for RP

Deal terms

- \$50m upfront
- \$12m convertible note
- \$190m of milestones
- Double-digit royalties

Exosome deals based on pre-clinical POC data*





Total: \$72.5m neuro-muscular targets

Codiak listed on Nasdaq in October 2020, raising \$83m

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Upfront: \$20m Total: \$1,230m neurological targets





Total: \$882m rare diseases







Upfront: \$56m Total \$1,076m cancer



SUMMARY – THE OPPORTUNITY





Major value creation opportunities over the coming year and beyond for hRPC – ReNeuron is well-funded to exploit these



Further data from expanded Phase 2a study of hRPC in RP to be presented later this year – data to date compare very favourably with other products in the field



Exosome programme being advanced through partnering while retaining rights



Potential of cell therapy in ophthalmology and increasing industry interest in exosomes underlined by recent high-value licensing deals and funding events



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