ReNeuron

CORPORATE PRESENTATION

October 2021

Olav Hellebø - Chief Executive Officer

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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 read-outs and exosome pre-clinical proof-of-concept data expected in Q1 2022

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PROPRIETARY PLATFORM TECHNOLOGIES

hRPC

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

Cryopreserved formulation allows global ship-and-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

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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 Q1 2022 • Pivotal trial to commence in late 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

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Lead Programme

hRPC in retinitis pigmentosa

RETINITIS PIGMENTOSA: AN UNMET NEED

RP is an inherited, degenerative eye disease[1,2,3]

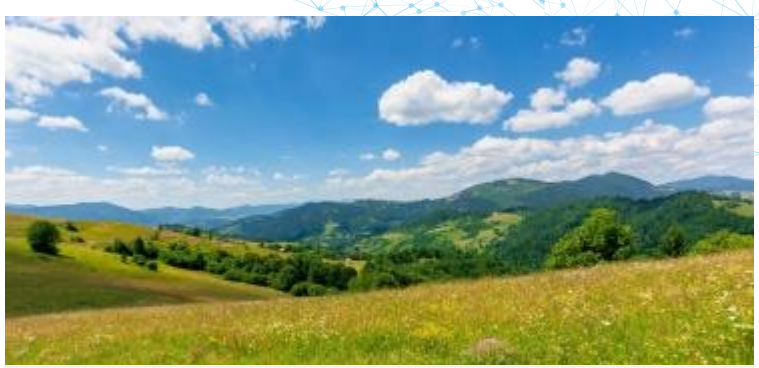
- Incidence of 1:4,000 in U.S. and worldwide

>100 genes identified containing mutations leading to RP[4]

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

Patients with all other types of RP (c98% of patients[5]) 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

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HUMAN RETINAL PROGENITOR CELLS (hRPC)

hRPC: allogeneic cell-based therapeutic approach to retinal disease

- 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

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

- Collaborations with Schepens Eye Research Institute (Harvard) and University College London
- Proprietary technology enabled development of GMP manufacturing process
- Cryopreserved formulation provides nine-month shelf life and enables local treatment worldwide

High commercial potential

- RP is a large orphan market.
  Attractive pricing precedent set by Luxturna
- Mechanism of action independent of genetic cause
- Commercially viable formulation

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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

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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

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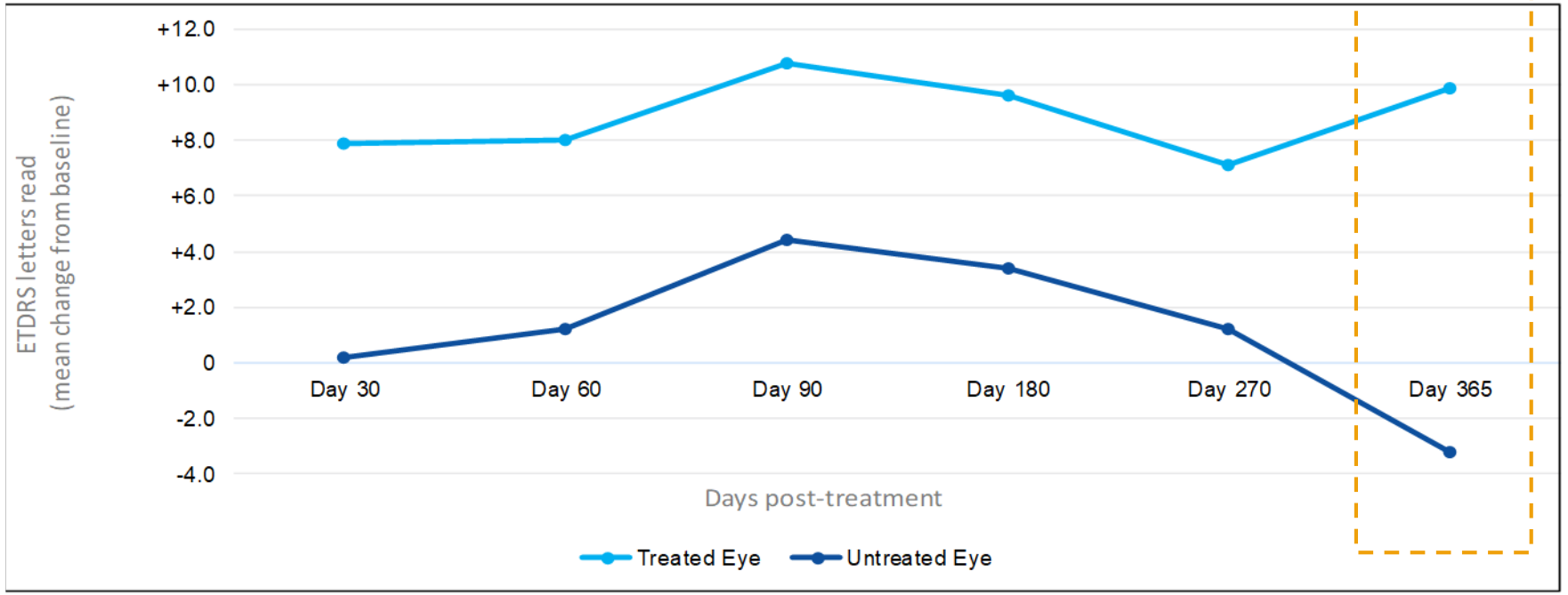
PHASE 2a EFFICACY RESULTS

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

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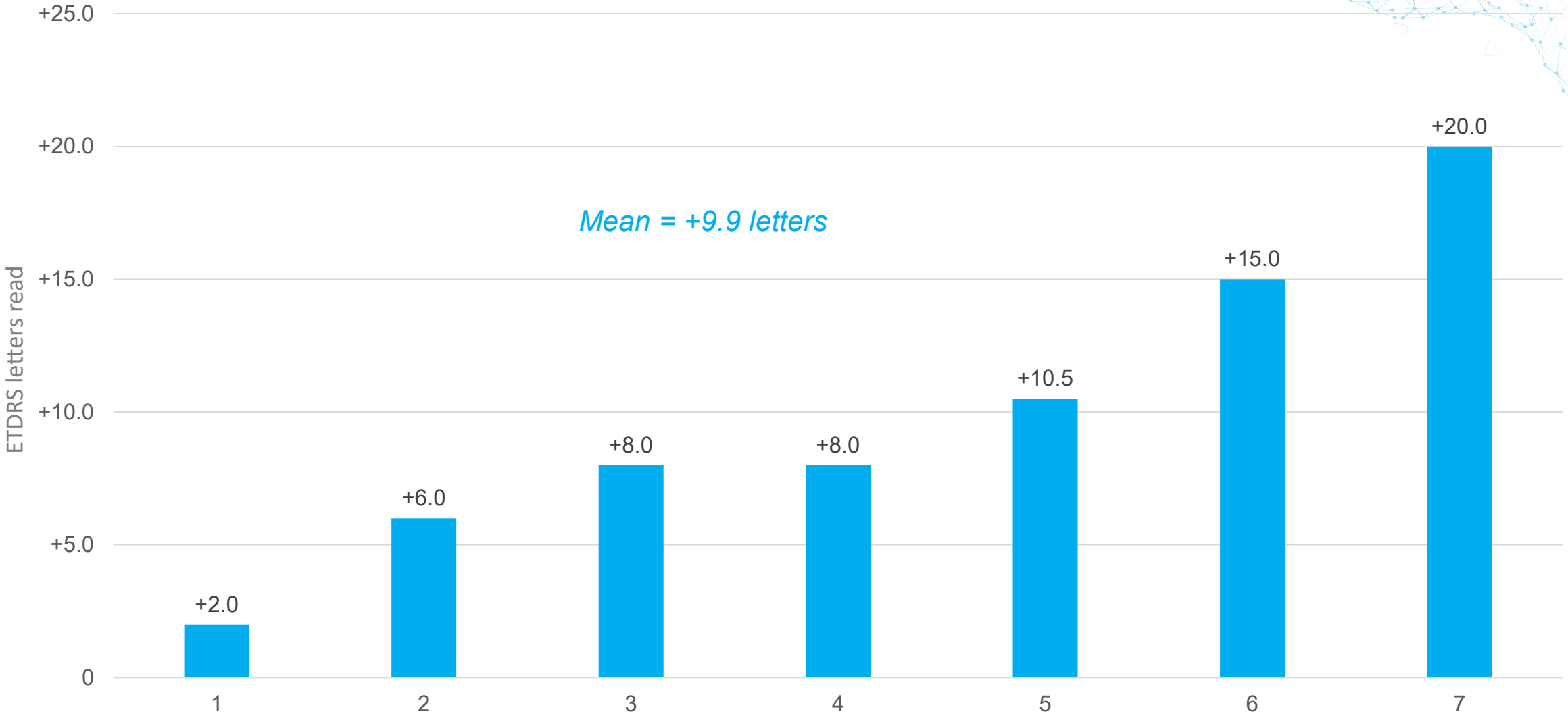
PHASE 2a EFFICACY RESULTS

INDIVIDUAL PATIENT IMPROVEMENTS AT 12 MONTHS

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



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CLINICAL DEVELOPMENT: PHASE 2a EXTENSION

Modifications to build on initial efficacy signal

Phase 2a Extension

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)

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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
- Remaining patients to be treated by the end of 2021

Further efficacy data to be available in Q1 2022

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)

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

Partnering strategy to be based on full Phase 2a data

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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	-

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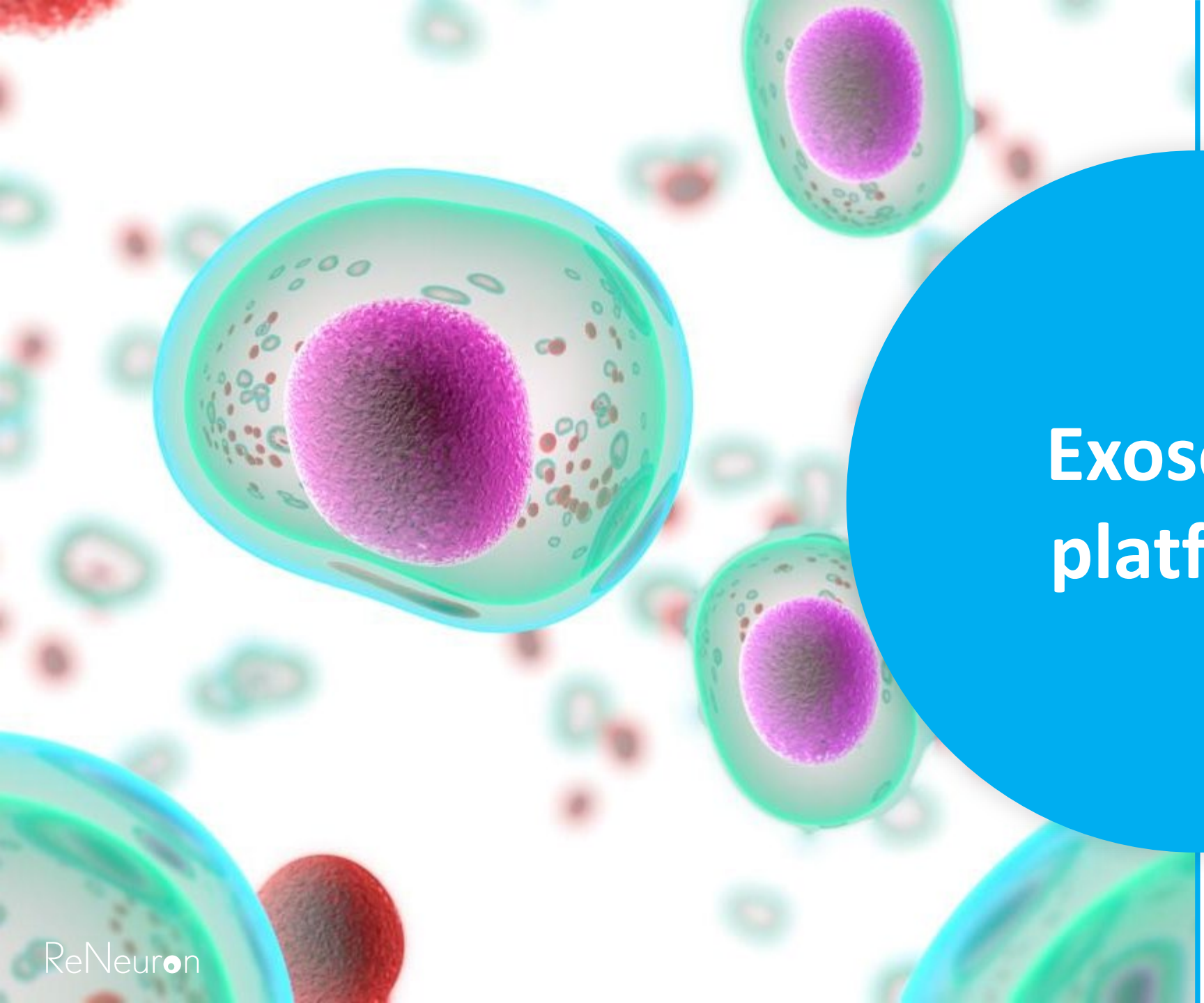
- Market capitalisation as at 05 July 2021

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



Exosome platform

EXOSOMES: BIOLOGICAL NANOPARTICLES

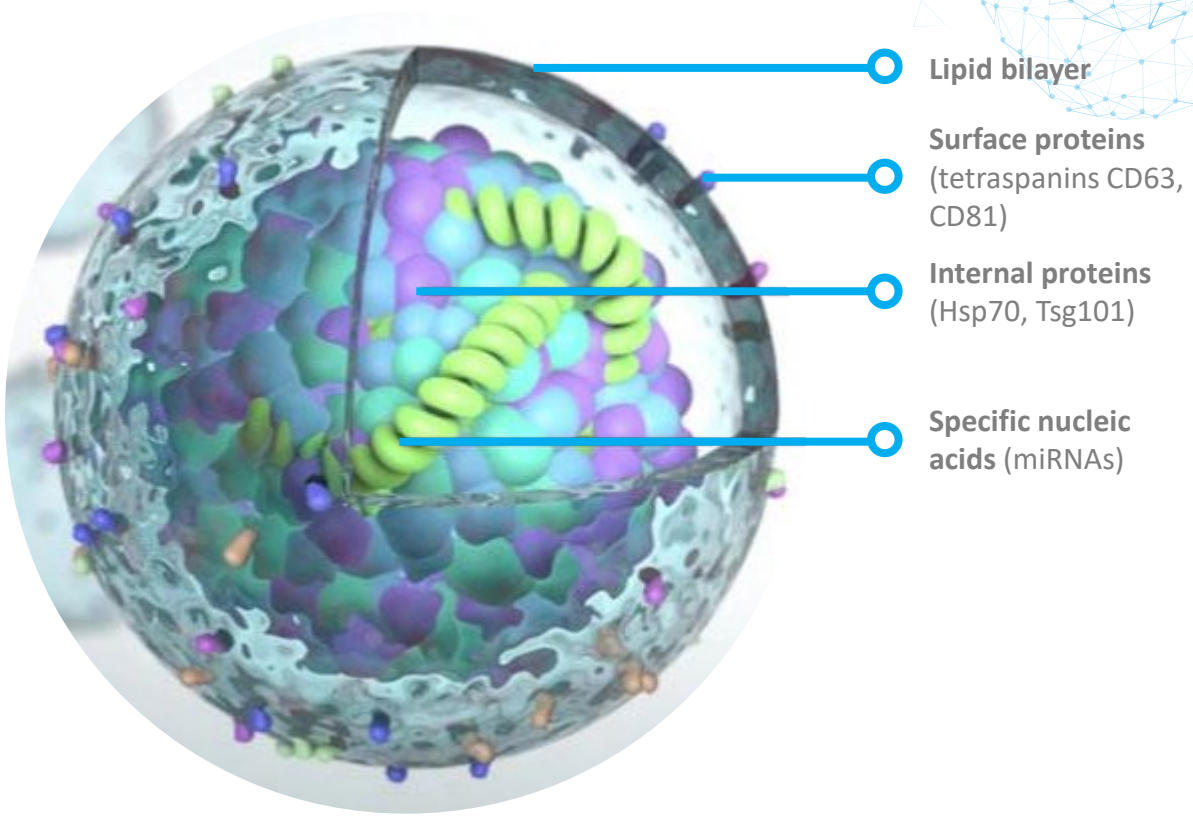
Nano-scale vesicles released by most cell types as a means of intercellular communication

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

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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

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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

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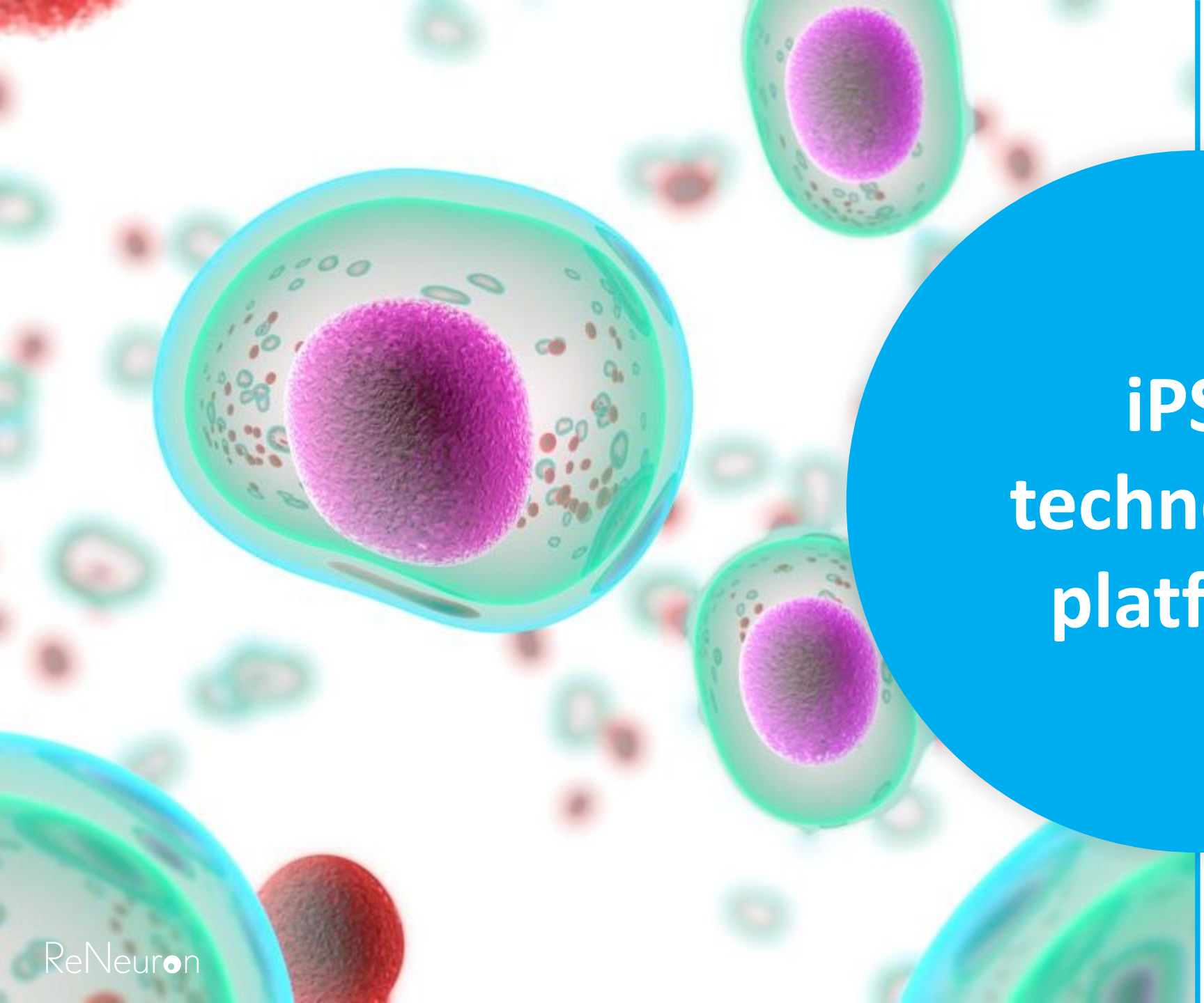


iPSC technology platform

INDUCED PLURIPOTENT STEM CELLS

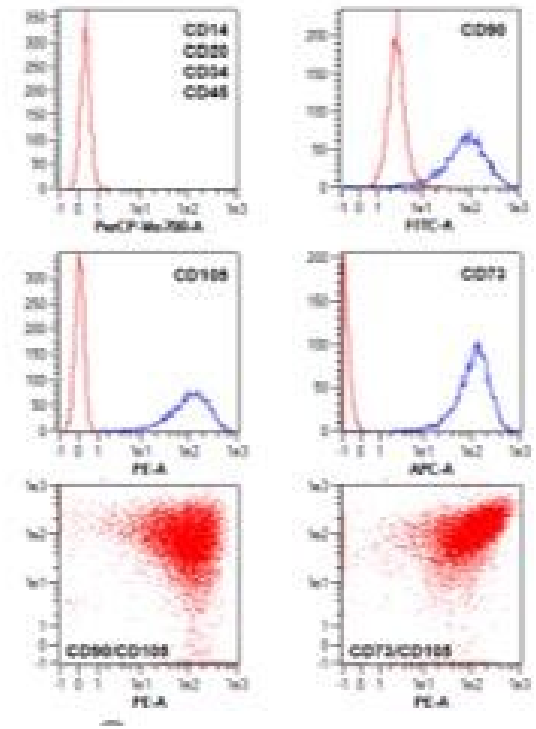
ReNeuron's iPSC platform technology is for reprogramming proprietary neural stem cells into a pluripotent state able to differentiate into any other form of stem cell

iPSCs retain the immortalisation characteristic of the stem cells from which they are derived, resulting in highly stable cell lines

Technology has the potential to lead to off the shelf therapeutics, such as T cells for CAR-T therapy

Also has the potential to produce exosomes with tissue-specific targeting ability

Two programmes – currently underway in the validation and characterisation stage



Conditionally immortalised derivatives from CTX-iPSCs

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Summary

VALUE REALISATION

Recent deals in cell therapy for retinitis pigmentosa and exosomes research

Santen deal based on Phase 2 data in RP*

jCyte / Santen

- jCyte Inc signed an ex-US licensing deal for its jCell product in May 2020 with Santen Pharmaceutical
- jCell 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*

CODIAK
SAREPTA THERAPEUTICS

Total: $72.5m
neuro-muscular targets

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

Lilly
evox THERAPEUTICS

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

evox THERAPEUTICS
Takeda

Total: $882m
rare diseases

CODIAK
Jazz Pharmaceuticals

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

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*Source – public announcements

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 in Q1 2022 – 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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BREADTH OF OPPORTUNITIES

	Product	Indication	Technology	Discovery	POC/Development	Milestones
Exosome Platform	ExoBDNF	Neurodegeneration (hearing loss, Parkinson's, Alzheimer's)	Expresses the neuroprotective growth factor BDNF (brain derived neurotrophic factor)			Pivotal pre-clinical 2020
	ExoKRAS	Oncology	Engineered to deliver siRNA (small inhibitory RNA) against KRAS G12D mutation			Pivotal pre-clinical 2020
	ExoSPIKE	COVID-19 prevention	Expresses SARS-Cov-2 spike protein for potential delivery of COVID-19 vaccines			Grant application – awaiting decision
	ExoXX	Diseases of the brain	Exosomes loaded with therapeutic of partners choice			Collaborations ongoing
iPSC Platform	CTX-HSC	Haematological cancers	Immortalised hematopoietic stem cells – for scaled production of allogeneic T or NK cells			Validation and further characterisation 2020
	CTX-PP	Diabetes	For generation of phenotypically stable human pancreatic progenitor cells			Validation and further characterisation 2020

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