

ReNeuron

Interim results Presentation

For the six months ended 30 September 2018

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Changing
patients' lives

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ReNeuron snapshot

Leading, clinical-stage cell therapy company

Expertise in developing allogeneic cell-based therapies

Multiple assets targeting areas of significant unmet need

65 employees with sites in UK and Boston, US

London AIM market listing: RENE.L

ReNeuron technology and pipeline

CTX platform

- Immortalised neural stem cell line
- 12 month shelf life
- Positive Phase IIa results in stroke at one year post-treatment

hRPC platform

- Human retinal progenitor cell line
- Sub-retinal delivery
- Cryopreserved formulation extends shelf-life

Exosomes platform

- Nano-sized vesicles from CTX cells
- Potential as drug load/delivery vehicle and as therapeutic agent

Programme	Pre-clinical	Phase I/IIa	Phase IIb	Next Milestone
CTX cells Stroke disability				Pivotal, multi-centre trial in US initiated (PISCES III) – data expected early 2020
hRPC cells Retinitis Pigmentosa				Top line Phase I/IIa data expected in mid-2019
Exosomes Drug delivery/therapy				Collaboration/partnering deals in 2019

Strategy – R&D

CTX platform

- Deliver placebo-controlled data in stroke disability using approvable primary endpoint
- Select patient population with best results from previous trials
- PISCES III designed to be valid as a pivotal study for regulatory purposes
- One further study expected for market approval

hRPC platform

- Build further safety data in RP using commercial formulation
- Treat patients with more intact retinas in order to assess efficacy potential
- Conduct controlled multi-centre Phase IIb trial in RP
- Assess CRD and other indications

Exosomes platform

- Pursue ExoPr0 as a drug delivery vehicle internally and through research collaborations and partnering
- Investigate ExoPr0 as therapy via collaborations

Discovery

- Build data set in CTX-iPS cell platform (e.g. allogeneic T-Cells)

Strategy – business development

CTX platform

- Global partnering following PISCES III results
- Consider option agreement or territory deals ahead of PISCES III completion

hRPC platform

- Partnering in US and Asia following efficacy signal from Phase I/II study
- Keep European rights for proprietary development and commercialization

Exosomes platform

- Broad collaborative/partnering approach
- Aggressive IP build and defence

Discovery

- Research collaborations to build CTX-iPS cell platform

Competitive landscape – cell therapy

SanBio (US/Japan)

- SB623 cells: modified mesenchymal stromal cells
- Phase IIa in stroke disability showed positive results in European Stroke Scale, NIHSS, and Fugl-Meyer out to 24 months post-treatment
- Controlled Phase IIb study in stroke disability to read out in 2019 (Fugl-Meyer as primary end point)
- Primary endpoint met in Phase II chronic Traumatic Brain Injury (TBI) study

jCyte (US)

- Human retinal progenitor cells injected into the vitreous in RP patients
- Phase IIa results showed improvement in visual acuity
- Ongoing, controlled, Phase IIb study in RP patients with BCVA between 20/80 and 20/800

Spark Therapeutics (US)

- Luxturna was approved by FDA in December 2017 for RP patients (biallelic RPE65 mutation-associated retinal dystrophy)
- Marketed with list price of \$850K (\$425K/eye) in the US
- EMA approval was granted November 2018
- Novartis has ex-US rights

Interim statement – operational highlights

CTX stem cell therapy candidate for stroke disability:

- Patient screening and enrolment commenced in Phase IIb clinical trial in the US
- Top line data from Phase IIb study expected in early 2020

hRPC stem cell therapy candidate for retinal diseases:

- Optimised formulation of the hRPC drug product developed and approved for use in ongoing Phase I/II clinical trial in retinitis pigmentosa in the US
- Patient dosing recommenced in Phase I/II study using optimised hRPC formulation
- Top line data from Phase I/II study expected in mid-2019

Exosome platform/discovery:

- Programme to be refocused on use of ExoPr0 as drug delivery vehicle, providing greater scope for potential near-term partnering deals
- ReNeuron has induced pluripotency in CTX cell line opening up a vista of opportunity in generating new allogeneic cell lines for out-licensing

Business development:

- Increased activity in the period due to third party interest in Company's core therapeutic programmes
- Active discussions ongoing with a number of commercial third parties

Interim statement – financial highlights

(£'m)	Six months ended 30 September 2018 (Unaudited)	Six months ended 30 September 2017 (Unaudited)	Year ended 31 March 2018 (Audited)
Revenue and other operating income	2.4	0.3	0.9
Research and development costs	(7.5)	(8.6)	(16.7)
General and administrative costs	(2.6)	(2.2)	(4.6)
Operating loss	(7.7)	(10.5)	(20.4)
Net finance income/(costs)	0.9	(0.5)	(0.6)
Tax credit	1.5	1.4	3.4
Loss for the period	(5.3)	(9.6)	(17.6)
<hr/>			
Net decrease in cash and deposits	(6.7)	(7.8)	(15.7)
Cash and deposits at start of period	37.4	53.1	53.1
Cash and deposits at period end	30.7	45.3	37.4

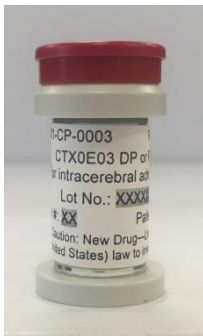


CTX cell line

CTX cell product

CTX - an allogeneic, cryopreserved human neural stem cell product

- Manufactured under cGMP with a 12 month shelf life
- Product to be readily ordered, shipped and stored at the hospital
- CTX platform allows for commercial scale manufacturing at attractive COGs
- Excellent safety profile - no immunogenicity issues post-administration



CTX delivered in cryo-shipper



Straightforward, controlled thawing at hospital site



Administer to patient 'on demand'

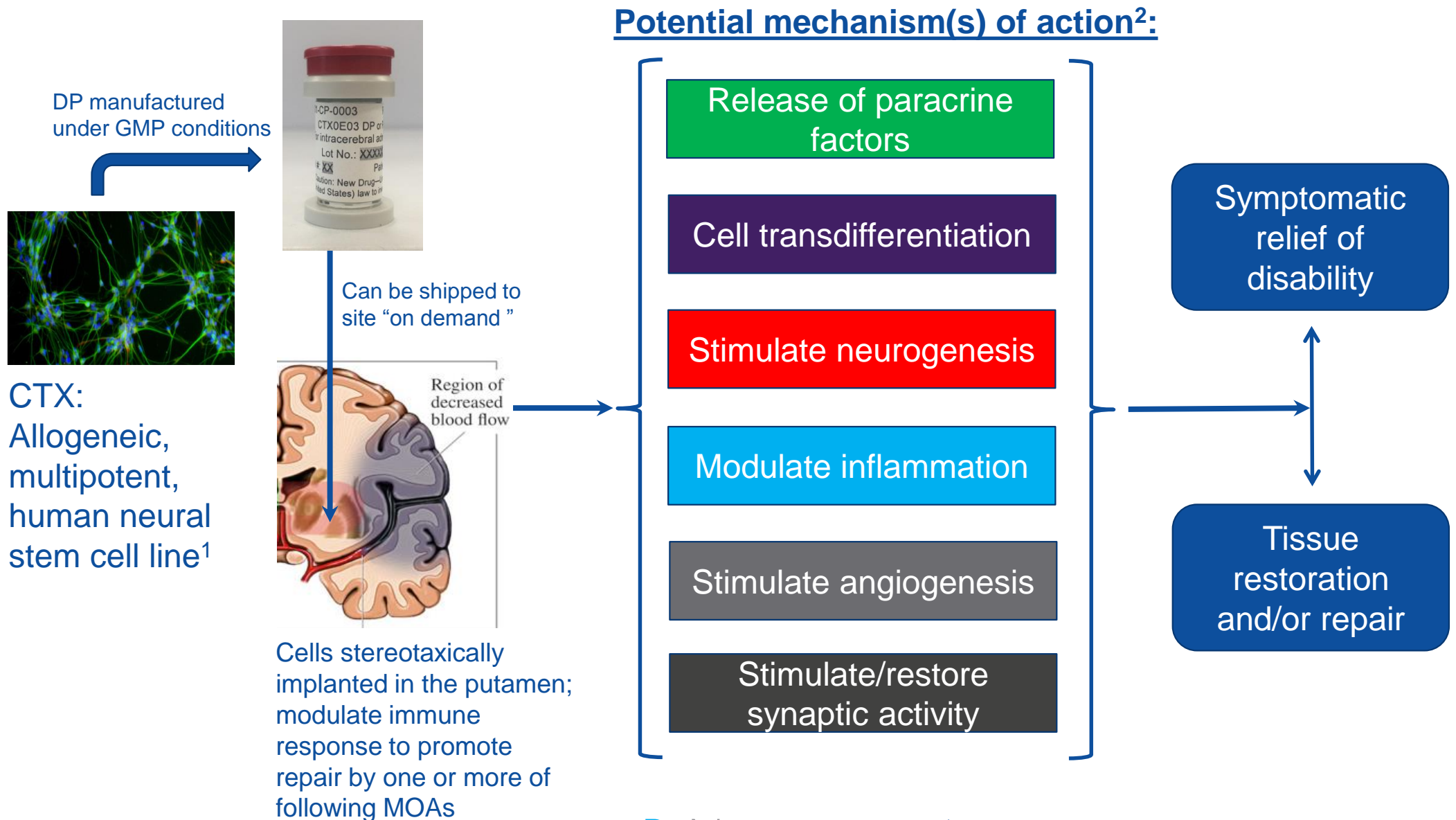
Similar to a conventional 'off-the-shelf' pharmaceutical / biologic drug

CTX for stroke disability: unmet medical need



- Stroke is leading cause of morbidity and long-term disability in US.¹
- Stroke costs are \$34 billion annually in US, including healthcare costs, medications, lost productivity.¹
 - Direct medical stroke-related costs are projected to triple between 2012 and 2030.¹
- Only one pharmaceutical treatment option available within 4.5 hours of stroke onset.²
- No treatment options available for stroke patients months to years later
 - Rehabilitation provides limited benefit – most in 1st month, less thereafter, very little beyond 6 months³
- CTX administration promotes repair in the damaged brain

CTX promotes anatomical plasticity in the brain



CTX:
Allogeneic,
multipotent,
human neural
stem cell line¹

CTX clinical development in stroke disability

- Completed PISCES trials

PISCES I:

- Phase I, first-in-human, safety study
- 11 disabled, stable stroke patients
 - 6 months to 5 years post-stroke
- CTX dose escalation (2, 5, 10, 20 million cells) administered by stereotactic, intracerebral injection
- No cell-related or immunological adverse events



CTX treatment is safe and well-tolerated

PISCES II:

- Phase II, single arm, open label
 - 20 million CTX cell dose
- 23 disabled, stable stroke patients
 - 2 to 12 months post-stroke
- Clinically meaningful improvements in disability scales were measured out to 12 months post-implantation
- No cell-related safety issues identified



Very promising results for chronic stroke disability; warrants a larger, randomised, placebo-controlled Phase IIb study

PISCES II positive efficacy

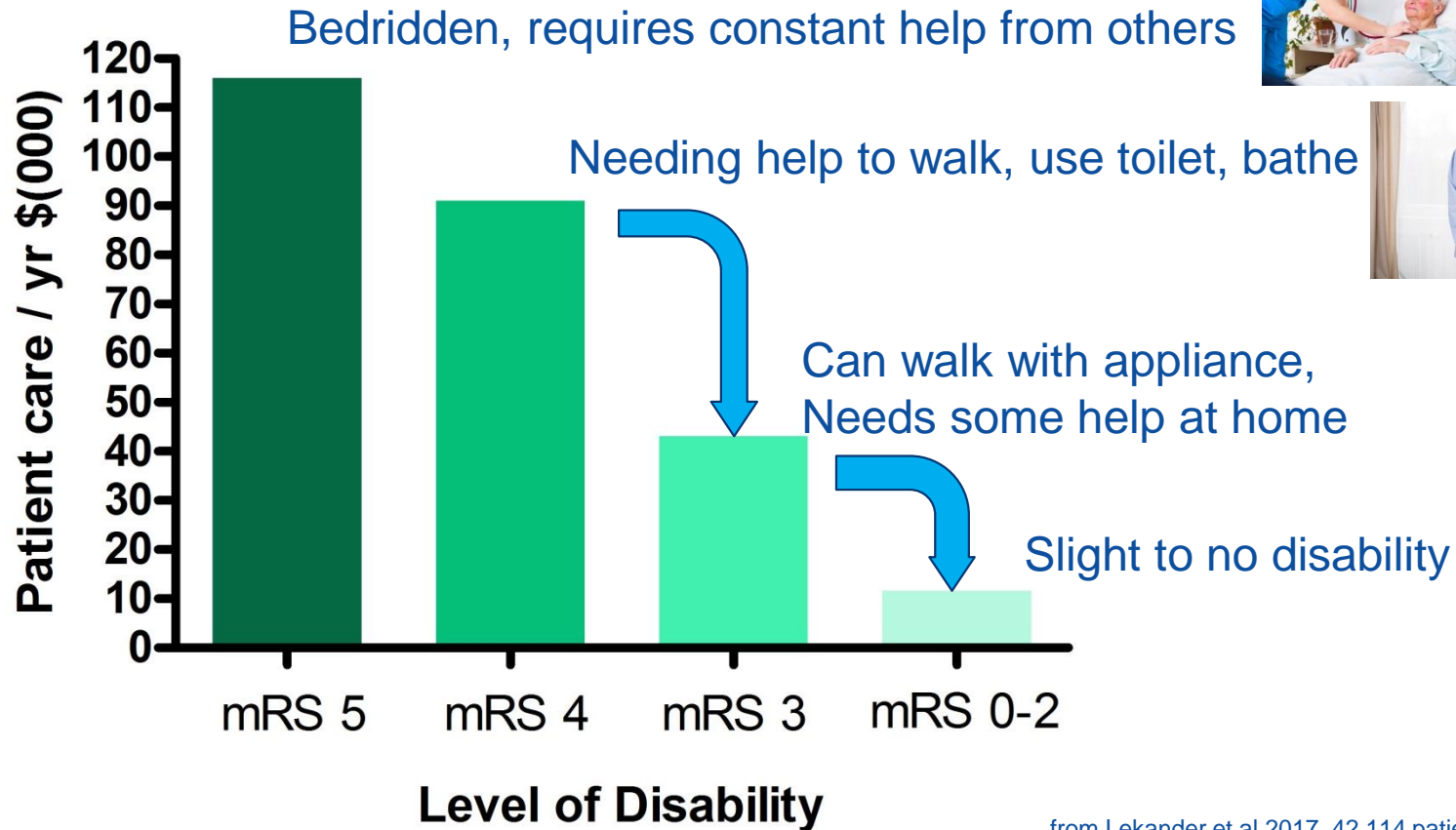
Modified Rankin Scale (mRS) – measure of functional disability

Total subjects			Patients with NIHSS upper limb score < 4 at baseline		Patients with NIHSS upper limb score = 4 at baseline	
Month	N	Responders* (%)	N	Responders* (%)	N	Responders* (%)
Baseline	23	-	14	-	9	-
3	23	7 (30.4%)	14	6 (42.9%)	9	1 (11.1%)
6	22	6 (27.3%)	13	5 (38.5%)	9	1 (11.1%)
12	20	7 (35.0%)	12	6 (50.0%)	8	1 (12.5%)

*number of subjects with ≥ 1 point improvement in mRS (% of N observed at day of visit)

Improvements in mRS greatest in subjects with residual movement of the affected arm (NIHSS UL <4)

Costs of disability – mRS scale



from Lekander et al 2017, 42,114 patients from 2007-2012
Costs from Sweden, translated into \$

Reductions in disability result in substantial reductions in patient care costs

PISCES III clinical trial



PHASE IIb
INVESTIGATION
OF STEM CELLS
IN STROKE

Primary Objective

- Assess efficacy of intracerebral CTX by change in degree of dependency and disability as measured by mRS

Primary Endpoint*

- ≥ 1 pt improvement from baseline in mRS at 6 months post-treatment

Secondary Endpoints* (1, 3, 6, 9, 12 months post-tx)

- Barthel Index (ADL independence)
- Timed Up and Go test (lower limb and trunk function)
- Chedoke Arm/Hand Activity Inventory (upper limb function)
- NIHSS
- Fugl-Meyer Assessment
- EQ-5D-5L (QoL)

Status

- 12 surgical sites and 21 patient assessment sites identified and approved for participation in study – initial sites activated
- Patient screening and recruitment underway
- CTX Drug Product batches in stock or scheduled for manufacture
- Top-line readout expected in early 2020

Subject Population:

- 1:1 randomisation to placebo (sham) surgery
- Age 35-75 inclusive
- Ischemic stroke that includes supratentorial region (CT/MRI confirmed)
- 6-12 months post-stroke
- mRS 3 and 4
- Some residual arm movement

* Based on FDA input



**Human Retinal Progenitor Cells
(hRPC)**

hRPC is a cell-based retinal therapy

hRPC: A unique and promising allogeneic cell-based therapeutic approach to retinal disease

- 1) *Capable of differentiating into retinal cells and integrating into retinal layers*
- 2) *Broad therapeutic potential across range of retinal diseases*

1

Long-standing collaboration with Schepens Eye Research Institute (Harvard), Mass Eye and Ear (Boston), and University College London (Moorfields)

2

Proprietary technology enabled development of GMP manufacturing process to support clinical application

3

Developed cryopreserved formulation to extend product's shelf life allowing for worldwide shipment on demand

Initially targeting inherited retinal degenerative diseases

hRPCs may restore lost vision associated with Inherited Retinal Disease (IRD)

Retinitis Pigmentosa:
primary loss of rod
photoreceptors



>100 genes identified containing
mutations leading to RP ¹

Cone Rod Dystrophy:
primary loss of cone
photoreceptors



>20 genes that can have mutations
leading to this condition ²

Therapeutic benefit of hRPC approach not dependent on genetic causes of IRD

¹<https://www.genome.gov/13514348/learning-about-retinitis-pigmentosa/>

²<https://www.fightingblindness.ie/eye-conditions/cone-rod-dystrophies/>

Retinitis Pigmentosa (RP)

- RP is an inherited, degenerative eye disease^{1,2,3}
 - Onset varies from early childhood to 20s/30s
 - Early stage main symptom is night blindness
 - Progressive loss of peripheral vision (ie tunnel)
 - Incidence of RP is 1:4000 in US and worldwide
- Orphan Drug Designation in EU and US
- FDA Fast Track Designation in US



NORMAL VIEW



VIEW WITH
RETINITIS PIGMENTOSA

www.eyehhealthweb.com/retinitis-pigmentosa

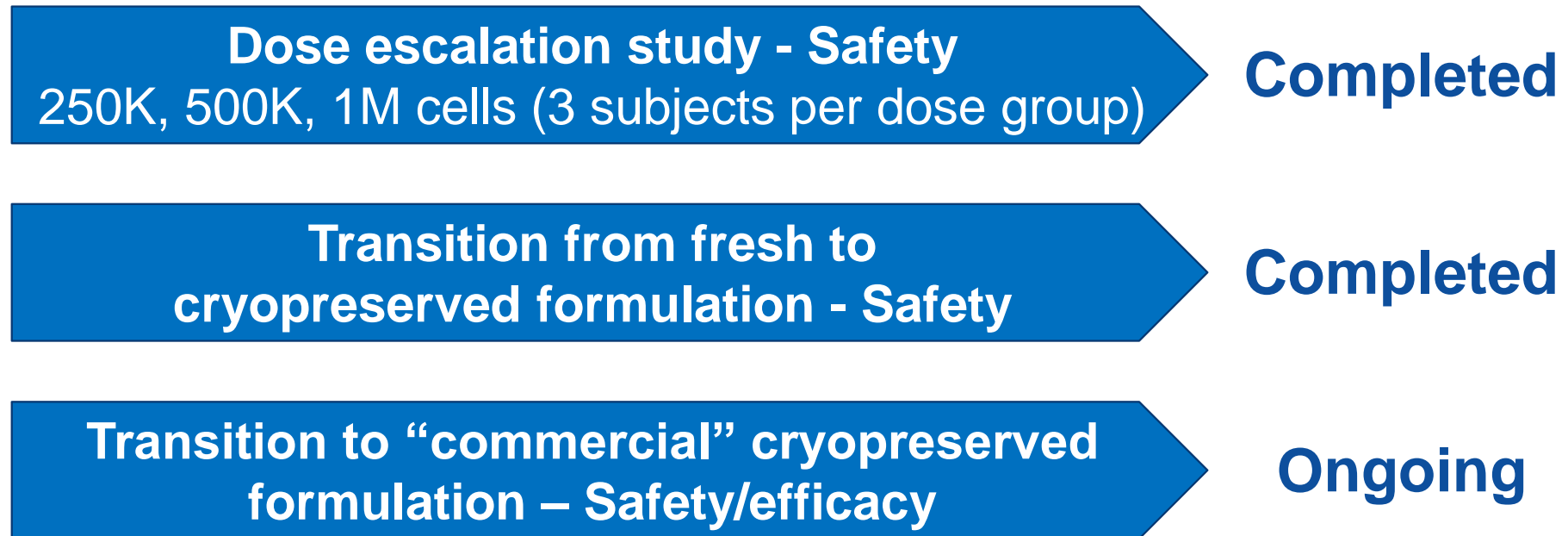
There is no treatment for the vast majority of patients with RP

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

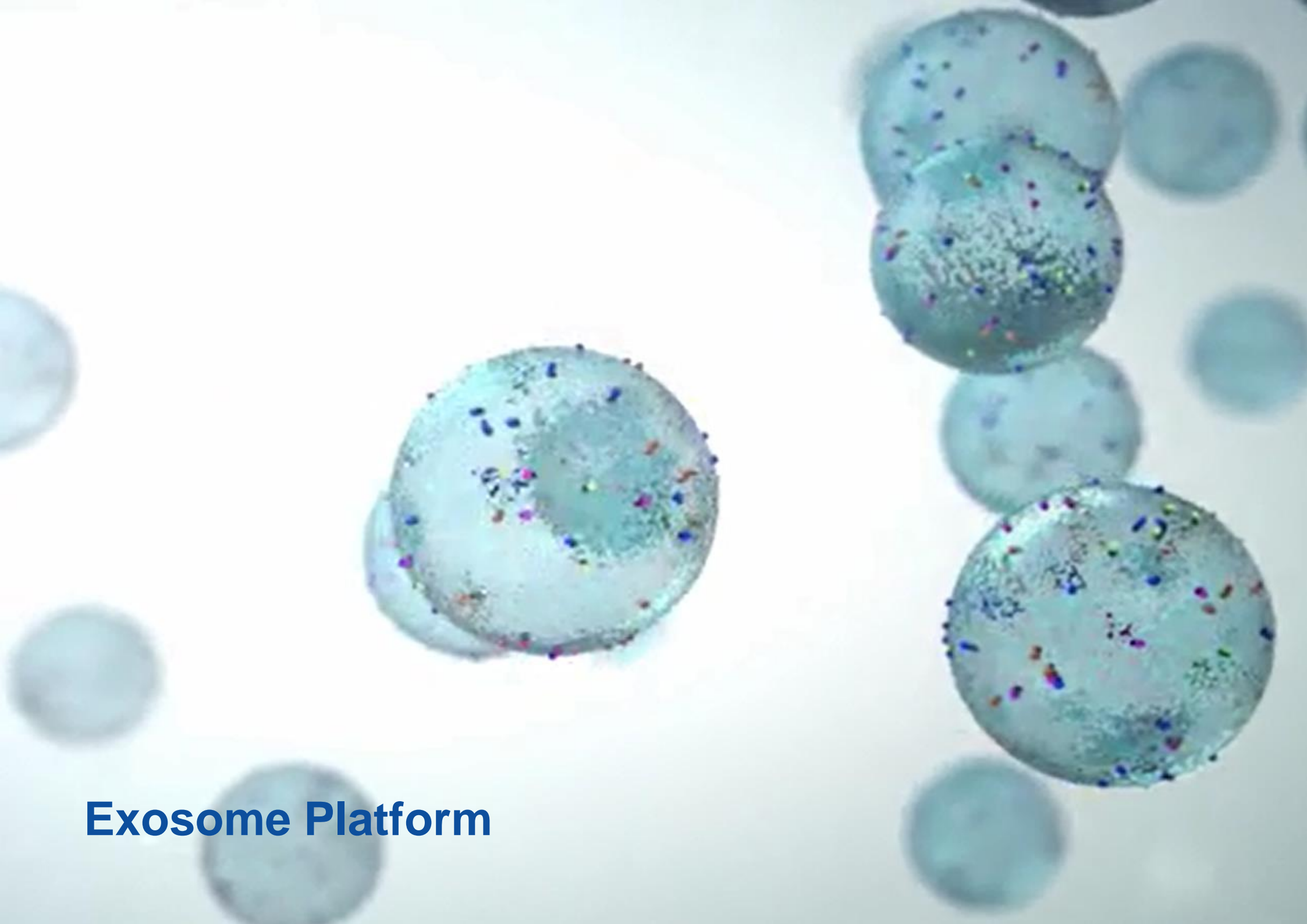
²https://nei.nih.gov/health/pigmentosa/pigmentosa_facts;

³NORD

US Phase I/IIa clinical trial

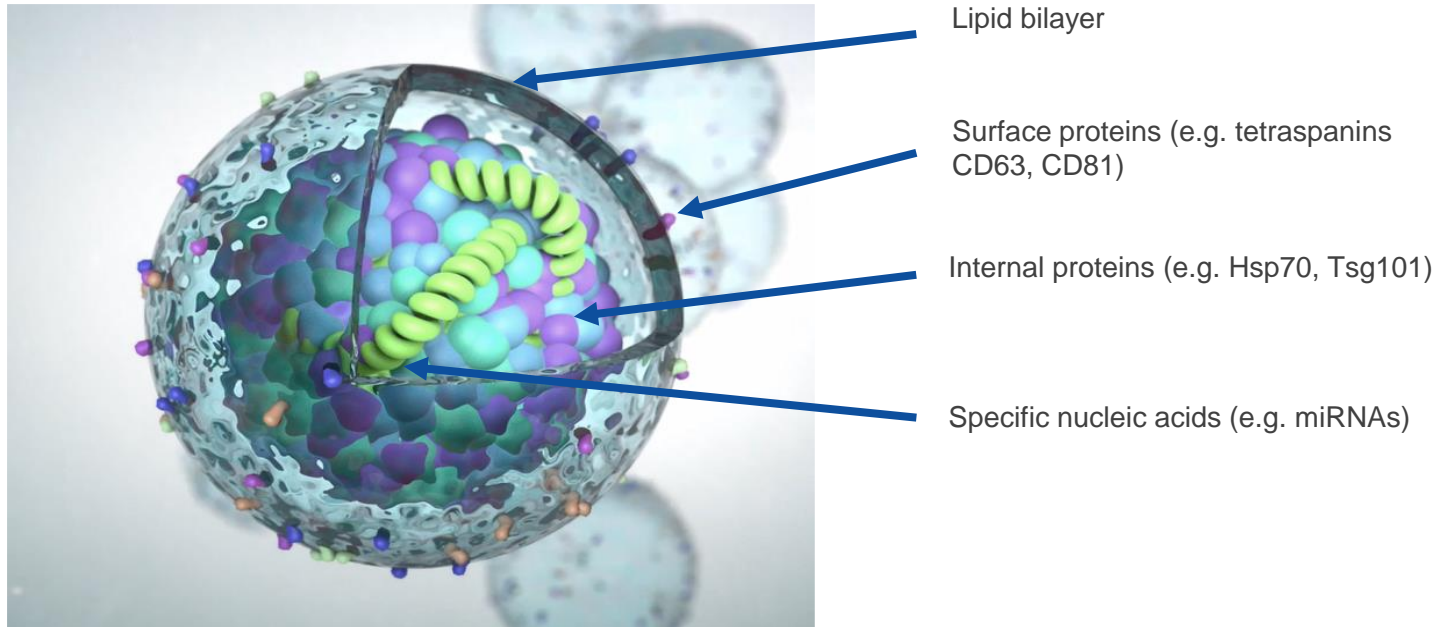


- RP subjects with low VA have been treated to date
- Treatments now commenced in RP patients with better sight to assess efficacy
- Top-line readout expected in mid-2019



Exosome Platform

Exosomes: biological nanoparticles



- Nano-scale vesicles (30-100nm) 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

Exosomes as a novel delivery vehicle

Exosomes (vs other delivery technology)

- Natural carriers of nucleic acids and proteins making them amenable for loading of complex, hard-to-deliver therapeutic agents
- Ease of bioengineering
- Low immunogenicity
- Intrinsically durable: Membrane texture order of magnitude harder than synthetic liposomes
- LNPs (lipid nanoparticles) are currently the most advanced delivery system, but:
 - Precise mechanism underlying LNP delivery not yet understood
 - Low efficiency (>90% degraded in lysosomes)
 - Significant inflammatory response

Advantages of CTX-derived exosome platform

ReNeuron's CTX-derived Exosomes

- Platform of endogenous high-yielding CTX cell line-derived exosomes
- Proven ability to load exosomes with miRNA and proteins
- Conditional immortalisation of CTX producer cell line ensures consistent exosome product
- Fully qualified xeno-free, optimised, scalable GMP process
- Established analytical package for in-process controls and batch-to-batch consistency
- Favourable distribution across the blood brain barrier
- Significant IP portfolio established
- CTX-derived exosomes can be modified to carry siRNA/mRNA/miRNA, or CRISPR/Cas9 proteins, small-molecule inhibitors and engineered to target particular tissues

Summary

- Global leader in cell-based therapeutics
- Allogeneic stem cell technology platforms – patented, scalable & cost effective
- Targeting diseases with large unmet medical needs
- Significant clinical milestones in stroke and retinal programmes during the next 18 months
- Near/medium term opportunities for value-generating partnering/collaboration deals

ReNeuron

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