ReNeuron

CORPORATE PRESENTATION

December 2020

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RENEURON TEAM AND KEY CLINICAL ADVISERS

Olav Hellebø

Chief Executive Officer

Olav has held leadership roles internationally at big pharma companies, including Novartis and Schering Plough, and biotechs including Clavis Pharma ASA. Product launches include the TNF-blocker Cimzia whilst at specialty biopharma business UCB

Michael Hunt ACA

Chief Financial Officer

Michael qualified at Ernst & Young after which he joined Bunzl plc before focusing on healthcare at Biocompatibles International plc and then ReNeuron. He sits on the board of the US-based Alliance for Regenerative Medicine and other industry bodies

Dr. Rick Beckman

Chief Medical Officer

After a career as an ophthalmologist in academics, then private practice, Rick moved into leadership roles at large companies including Allergan, Alcon and BD. He then moved on to serve as CMO at ophthalmology-focused biotechs including Neurotech, Ophthotech, and Clearside.

Dr. Tim Corn

Chairman

Dr. Mike Owen

Non-executive Director

Sir Chris Evans Non-executive Director

Mark Evans

Non-executive Director

Clinical Advisors

Dr Jason Comander

Associate Director of the Inherited Retinal Disorders Service at Massachusetts Eye and Ear Infirmary and Assistant Professor of Ophthalmology at Harvard Medical School

Prof. Robert MacLaren

Professor of Ophthalmology, University of Oxford, directs research into new treatments for blindness. Co-founded Nighstar Therapeutics, which was acquired by Biogen.

Dr Timothy Stout

Chair of the Ophthalmology Department and Director of the Cullen Eye Institute at Baylor College of Medicine.

Dr Jordi Monés

Macula and Vitreorretinal Specialist and Researcher. Director of the Institut de la Màcula and the Director, Principal Investigator and one of the founder governors of the Barcelona Macula Foundation: Research for Vision.

Dr Karl Csaky

T. Boone Pickens Director, Molecular Ophthalmology Laboratory and Clinical Center of Innovation for Macular Degeneration.

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

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

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

Well-funded, with multiple value inflection points expected in the next 12 months, including extended RP Phase 2a clinical data read outs and exosome pre-clinical proof-of-concept data

PROPRIETARY PLATFORM TECHNOLOGIES

hRPC	Exosome Platform	iPSC Platform	CTX Cells
Human Retinal Progenitor Stem Cells with sub-retinal delivery enabling engraftment	High-yielding neural stem cell derived exosomes	CTX-based induced pluripotent stem cell platform	Immortalised neural progenitor stem cell line
Cryopreserved formulation allows global ship-and-store	Proven ability to load exosomes with siRNA, miRNA and proteins	Neural stem cells engineered into other forms of stem cells while preserving the immortalisation	Positive clinical data in stroke disability. Potential in Huntington's disease, TBI and other indications
Positive early Phase 2a data in ongoing retinitis pigmentosa study	Favourable distribution of exosomes across the Blood Brain Barrier	Potential to create allogeneic CAR-T cell therapies and pancreatic islet cells	Out-licensing strategy
Partnered with Fosun Pharma for China	Potential as drug load/delivery vehicle and as a therapeutic. 3 pharma collaborations ongoing		Partnered with Fosun Pharma for China
Example Peers: nightstar THERAPEUTICS, Spark THERAPEUTICS, jCyte	CODIAK, evox THERAPEUTICS		

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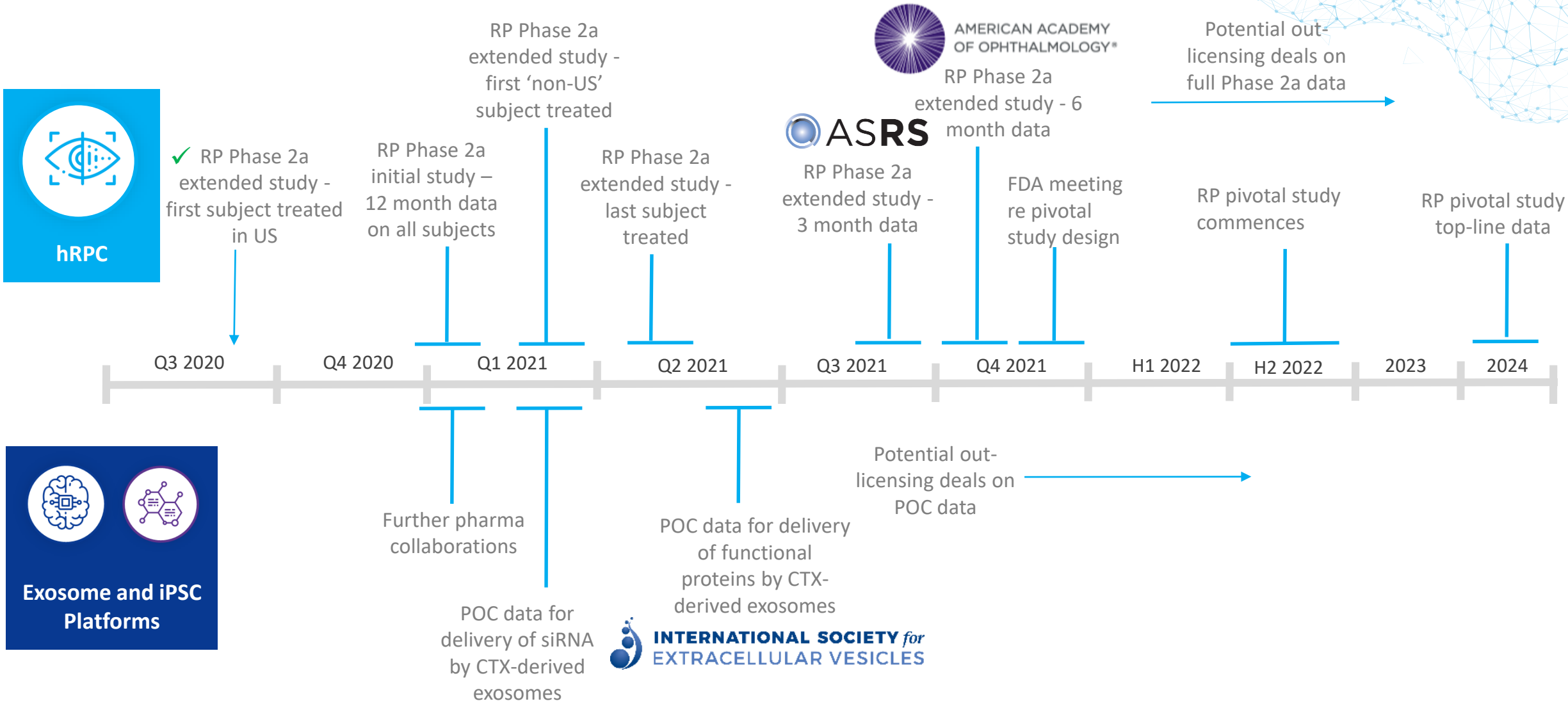
INTERNAL RESEARCH AND DEVELOPMENT PIPELINE

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 over next twelve months • Pivotal trial to commence in H2 2022, subject to Phase 2a data
Exosome platform	Neurodegeneration, Oncology, Vaccines (e.g. COVID-19)				• Proof of concept data from current research collaborations expected in H1 2021 • Additional collaborations expected over the next 12 months
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

MATERIAL VALUE INFLECTION POINTS TARGETED OVER NEXT 12 MONTHS AND BEYOND(1)



(1) Indicative clinical timelines subject to successful recruitment of patients

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

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

Existing Clinical Sites	Massachusetts Eye & Ear Infirmary, Boston	Retinal Research Institute, Phoenix

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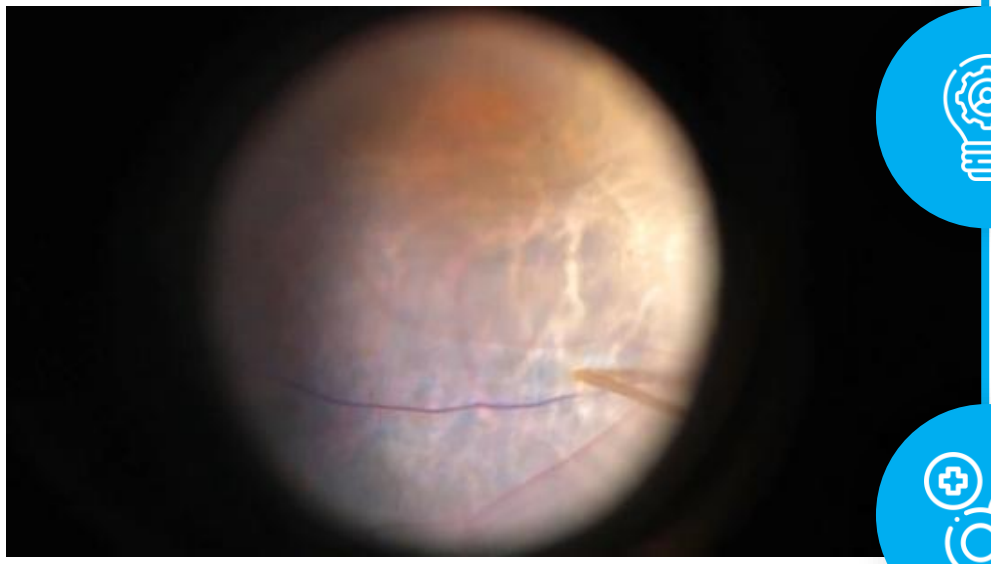
SURGICAL TECHNIQUE

Sub-retinal Injection



Well established technique used commercially with Luxturna®

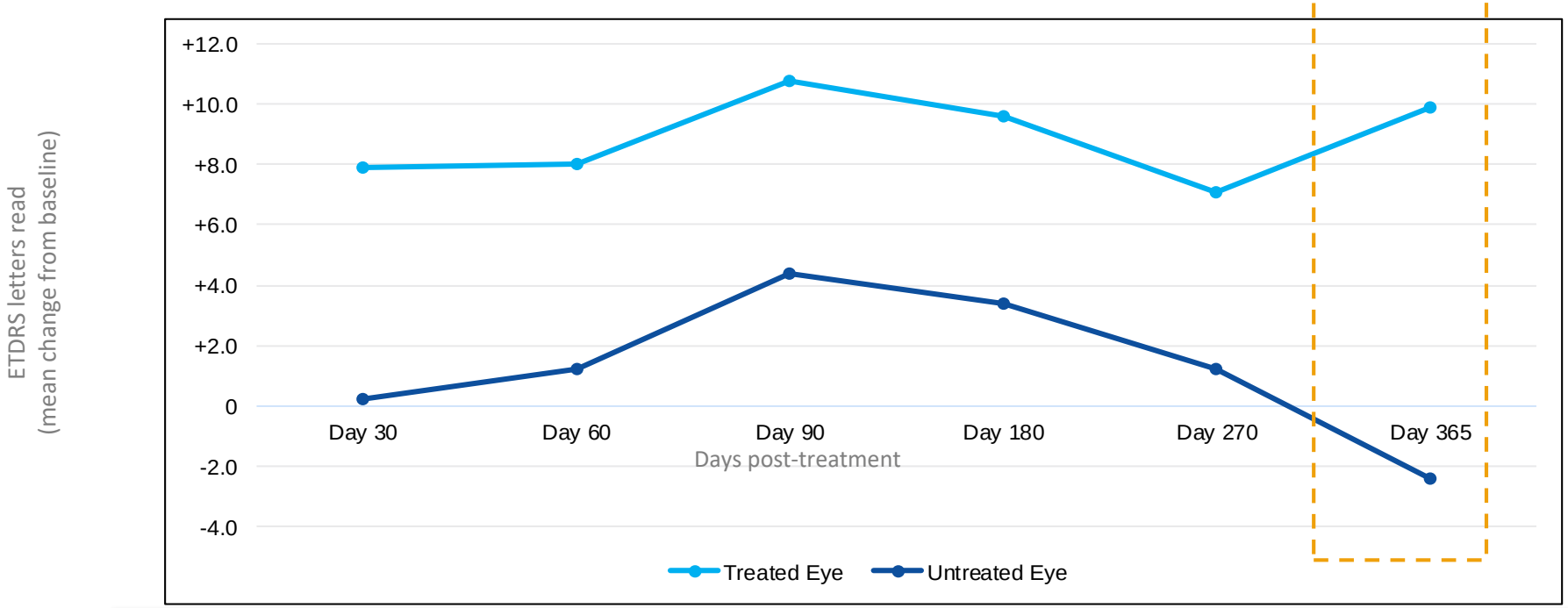
Allows correct anatomic placement of cells for integration into the retina

- 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

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	-2.4
Difference	+7.7	+6.8	+6.4	+6.2	+5.9	+12.3



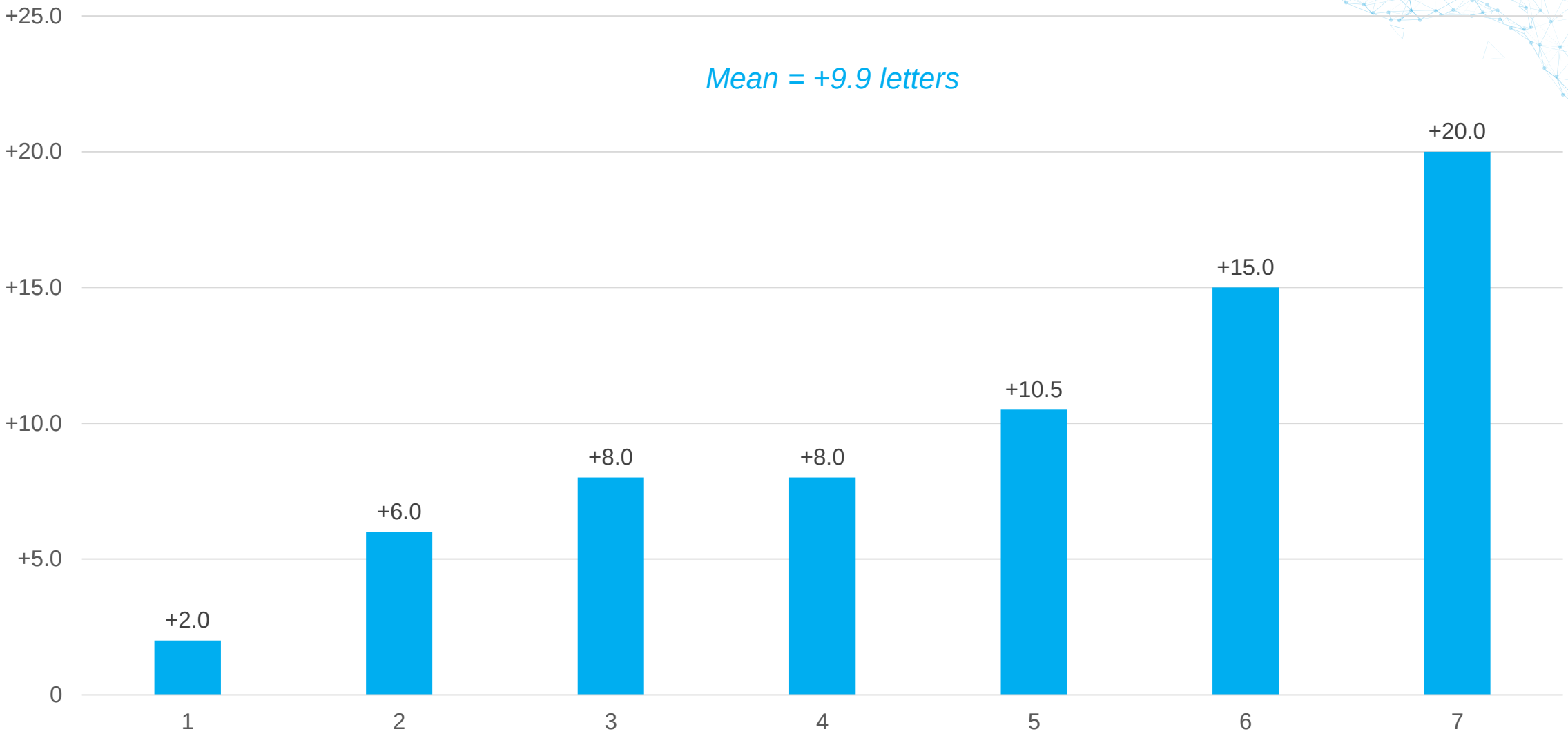
Additional Notes:

*excluding 1 patient (6003) with surgery-related vision loss

**Two patients have so far been assessed at 18 months. One patient has gained 17 letters from baseline in the study eye and one letter in the non-study eye. The second patient has gained six letters from baseline in the study eye and 22 letters in the non-study eye.

INDIVIDUAL PATIENT IMPROVEMENTS AT 12 MONTHS

ETDRS change from baseline 12 months post treatment (n=7)



CLINICAL DEVELOPMENT: PHASE 2A EXTENSION

Modifications to better hone 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)	Two further sites planned, one in Europe and one in the US

RETINITIS PIGMENTOSA: THERAPY LANDSCAPE

Company	Technology	Stage	Comment
ReNeuron (AIM, market cap: £26m*)	Cell therapy	Phase 1/2a	Cryopreserved formulation
jCyte Inc (US, private)	Cell therapy	Phase 2b	Not cryopreserved; used to date in California and Massachusetts
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 $585m*)	Gene therapy	Phase 1/2	-
AGTC (Nasdaq, market cap $118m*)	Gene therapy	Phase 1/2	-

* Market capitalisations as at 7 December 2020
** www.nice.org.uk/guidance/hst11/chapter/2-The-condition

RETINAL PLATFORM NEXT STEPS

Material newsflow and value inflection points over the next 15 months and beyond

Collect long term data in normal dose subjects

- Most patient visits restarted post-Covid restrictions
- All 22 patients will be followed to at least 24 months post treatment

Recruit high dose expansion study

- First patient treated September 2020
- Enhancements in patient selection, dose, surgical technique and efficacy assessments

Further efficacy data to be presented at retinal conferences over the coming twelve months

- AAO/ARVO are the key conferences in ophthalmology
- 3 months data on all 9 Phase 2a extension studies to be presented at ASRS in July

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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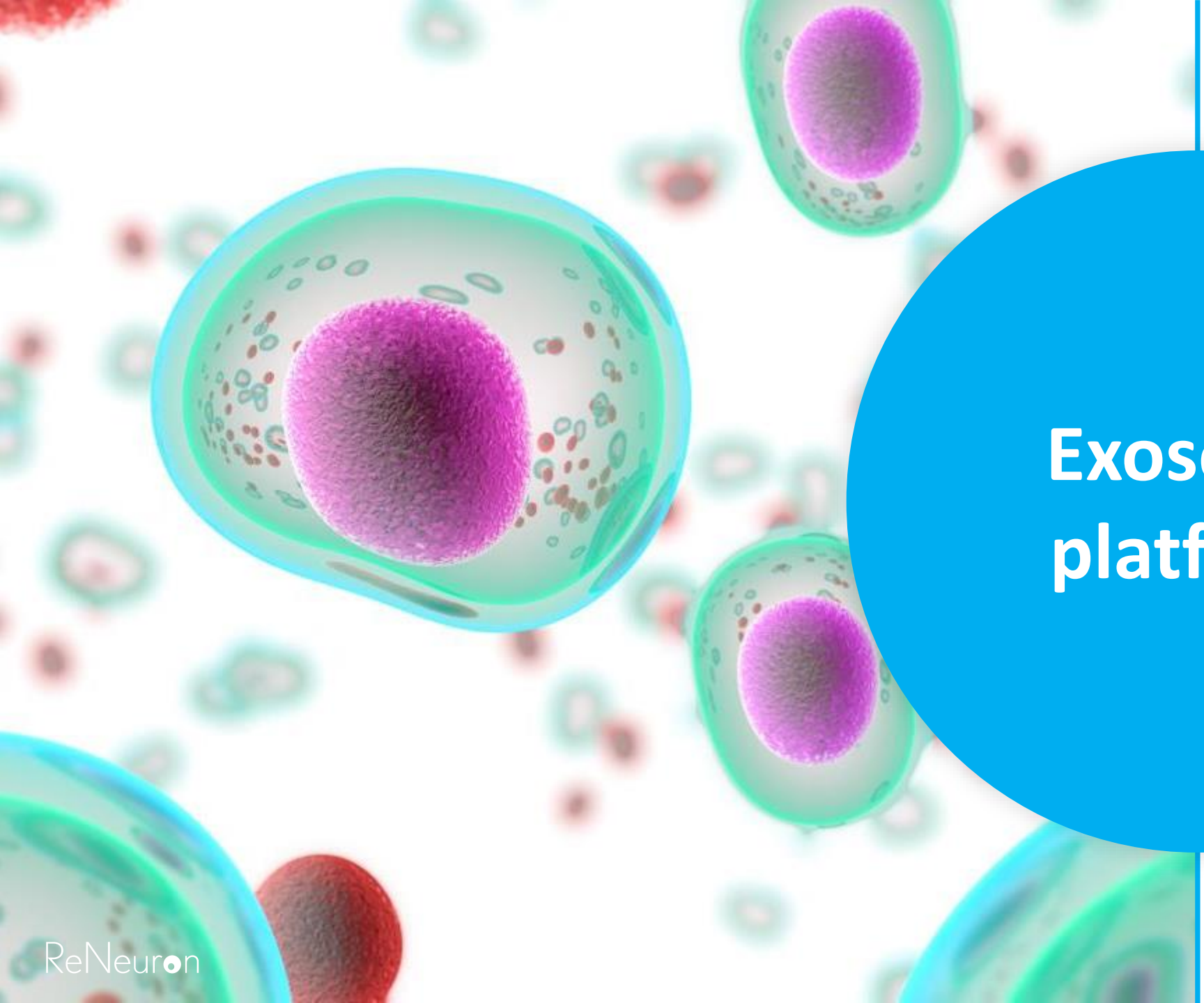


Exosome platform

EXOSOMES: BIOLOGICAL NANOPARTICLES

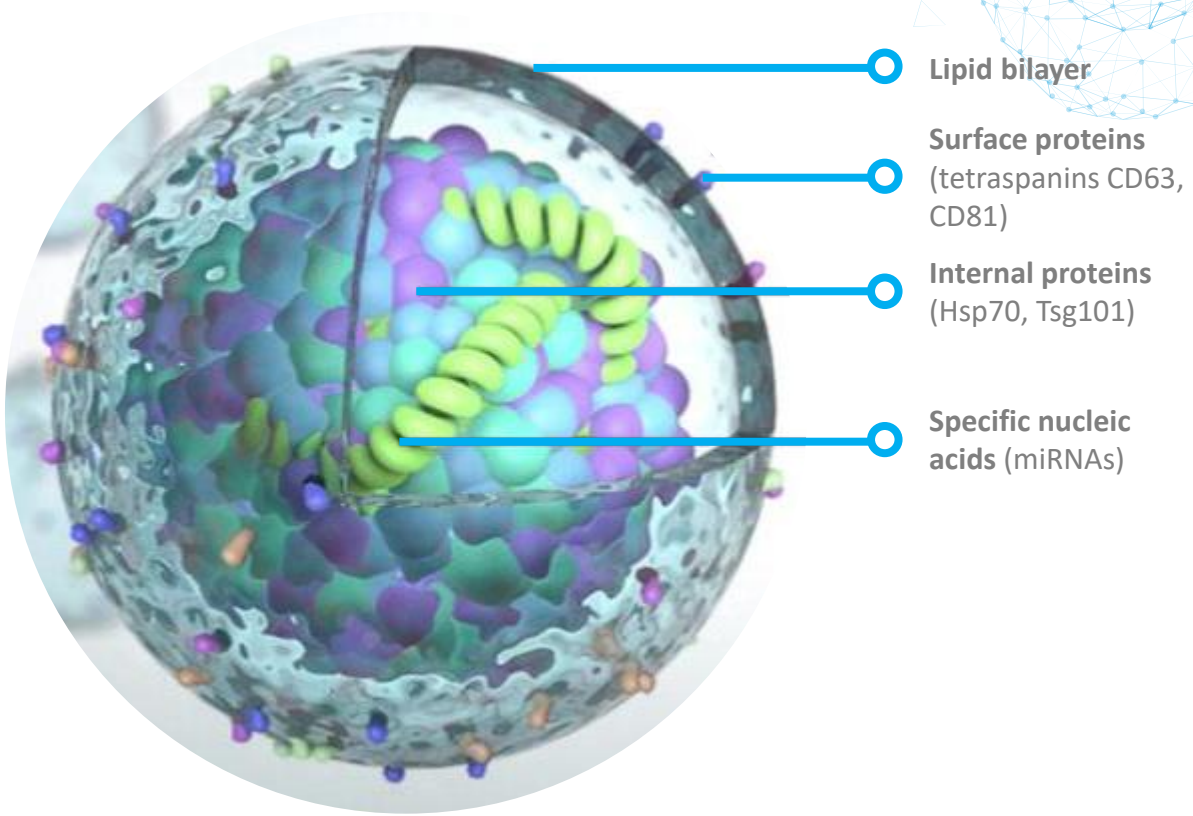
Nano-scale vesicles released by most cell types as a means of intercellular communication

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
- Collaborations in place and further ones under negotiation



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 H1 2021

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

Significant research collaborations ongoing

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

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

ReNeuron owns equal co-development rights to new therapeutic modalities

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*Source – public announcements

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

*Source – public announcements

SUMMARY

Major value creation opportunities in the coming 12 months for hRPC

New data from expanded Phase 2a study of hRPC in retinitis pigmentosa to be presented at major conferences next year

Competitor data support ReNeuron's approach in RP

Potential of cell therapy in ophthalmology underlined by recent JCyte/Santen Pharmaceutical licensing deal

$252m in upfronts and milestones for ex-US rights plus double digit royalties

Exosome programme being advanced through partners while retaining rights

High level of industry interest in exosomes reflected in recent licensing deals

Deals totaling more than $2bn in upfronts and milestones based on proof of concept data

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