ReNeuron

pioneering stem cell therapeutics

Innovators and Investors Forum

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Company overview

- AIM-listed company (approx £95m market cap), a global leader in cell-based therapeutics
- Differentiated allogeneic (off-the-shelf) stem cell technology
- Significant indications:
  - Blockbuster potential in Stroke Disability and Critical Limb Ischaemia;
  - Orphan/Fast Track status for Retinitis Pigmentosa (RP)
- Clinical-stage therapeutic portfolio
  - Retinitis Pigmentosa: US Phase I/II study open for patient enrolment
  - Stroke disability: Phase II study ongoing
  - Critical limb ischaemia (CLI): Phase I study ongoing
- At forefront of emerging field of exosome-based nanomedicine

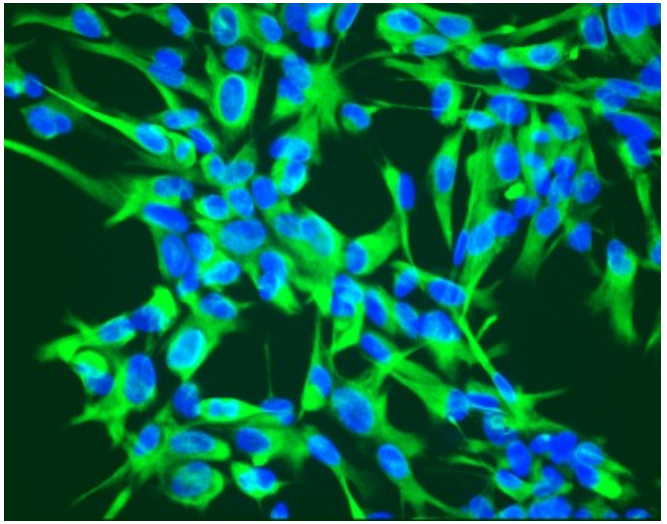
Well backed and well funded

- Backed by major generalist and specialist life science institutional investors:
  - Woodford Investment Management 35.5%
  - Wales Life Science Fund 9.5%
  - Invesco 9.3%
  - Aviva 6.3%
  - Abingworth 3.0%

- £72m cash on balance sheet (as of Sep 30, 2015), and no debt, providing three years of runway to potentially achieve:
  - Pivotal (pre-market) clinical read-outs in disability from stroke and RP
  - Phase II clinical proof-of-concept read-out in CLI
  - Exosome nanomedicine programme completes first-in-man study

Why stem cells?

- Stem cells are nature's repairing cell. They exist in most organs and tissues and help restore them after injury or disease
- Previously difficult to scale up commercially, stem cells can now be manufactured to clinical grade standards and developed as therapies
- Unlike pharmaceuticals, stem cells offer the potential for one-off treatments or even cures for chronic disabling diseases
- Stem cells can both replace damaged cells or deliver signals that restore normal tissue function after disease or injury



Unique Platform Technologies

One single stem cell

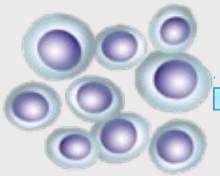
***CTX* cell line**

***CTX* platform**

Clinical and pre-clinical pipeline in vascular and neurological indications

Exosome platform

Potential to broaden therapeutic pipeline beyond cell-based programmes



hRPCs

Retinal platform

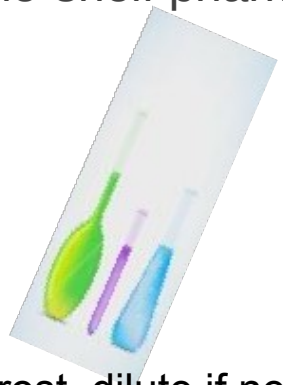
Targeting retinal degenerative diseases

In-licensed technology (Harvard, Boston)

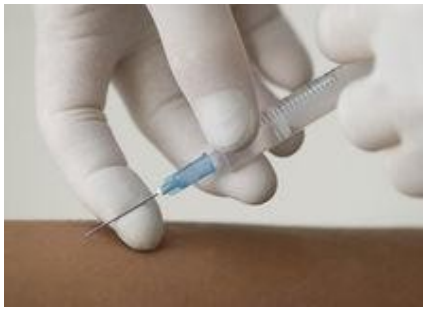
CTX cell product

- *CTX* - a GMP validated, cryopreserved human neural stem cell product
- 6 months shelf life
- Allows product to be readily shipped and stored at the hospital
- Closer to a conventional off-the-shelf pharmaceutical/biologic drug

CTX delivered in Cryo-shipper



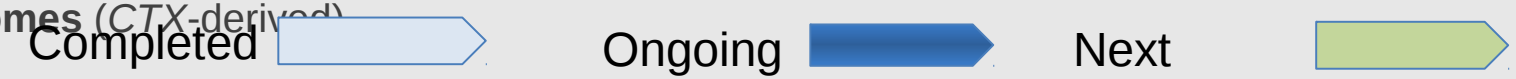
Defrost, dilute if necessary with excipient and invert to mix



Administer to patient “on demand”

Therapeutic pipeline

Programme	Pre-clinical	Phase I/II	Pivotal trial	Marketing Authorisation
***CTX* for motor disability from stroke**				
***CTX* for critical limb ischaemia**				
hRPC for retinitis pigmentosa				
Exosomes (*CTX*-derived)				



Retinitis pigmentosa

- RP is an inherited, degenerative eye disease

- Causes severe vision impairment and often blindness
- Incidence of RP is 1:4000 in the US with an estimated treatment population of 275,000 in the US and EU

- First therapeutic target for hRPCs

- Orphan Drug Designation in EU and the US & Fast Track Designation in US

- Phase I/II study open for enrolment in the US

- Pivotal Phase II/III planned to commence in 2017

RP vision

Motor disability from stroke

- Stroke is the single largest cause of adult disability
- According to World Health Organisation, each year, approx. 15 million people suffer their first ischaemic stroke
- Annual health/social costs: >$70 billion in the US
- No pharmaceutical treatment options available beyond 4 hours
- Pre-clinical efficacy/MOA of *CTX* cells demonstrated in multiple published studies*
  - Multi-modal MOA: angiogenesis, neurogenesis, immune modulation

- **Target is to improve recovery in disabled stroke survivors**

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- ** Smith et al: Stem Cells. 2012, Hassani et al: PLOS1 2012; Hicks et al: Cell Transplantation 2013*

Disability from stroke: clinical development plan

- UK Phase I study completed in 11 disabled stroke patients

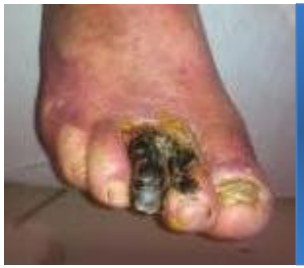
- Single, straightforward neurosurgical procedure
- No cell-related or immunological adverse events
- Encouraging results across multiple efficacy measures

- UK Phase II study ongoing (n=21)
  - Action Research Arm Test (ARAT) added as efficacy end-point (see image)
  - Phase II data: H1 2016

- Design controlled pivotal Phase II/III study based on t
  Phase II data
  - Phase II/III planned to commence H2 2016

Critical limb ischaemia

- Loss of blood flow to lower limb – common in diabetics
  - 160,000 legs amputated p.a. due to CLI in US alone
  - Large economic burden
  - Revascularisation surgery is treatment of choice (20-50% ineligible)
  - UK Phase I study ongoing – data H1 2016
  - Phase II study planned to commence H2 2016

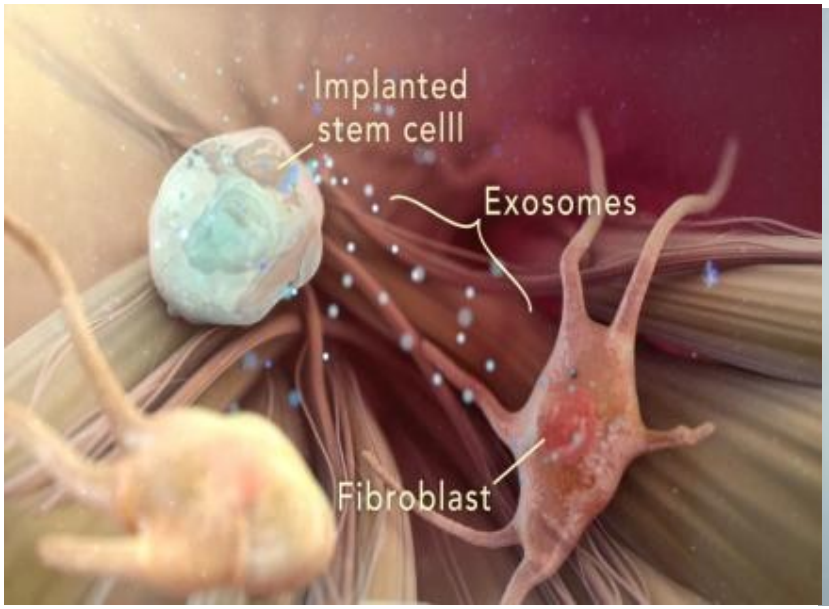


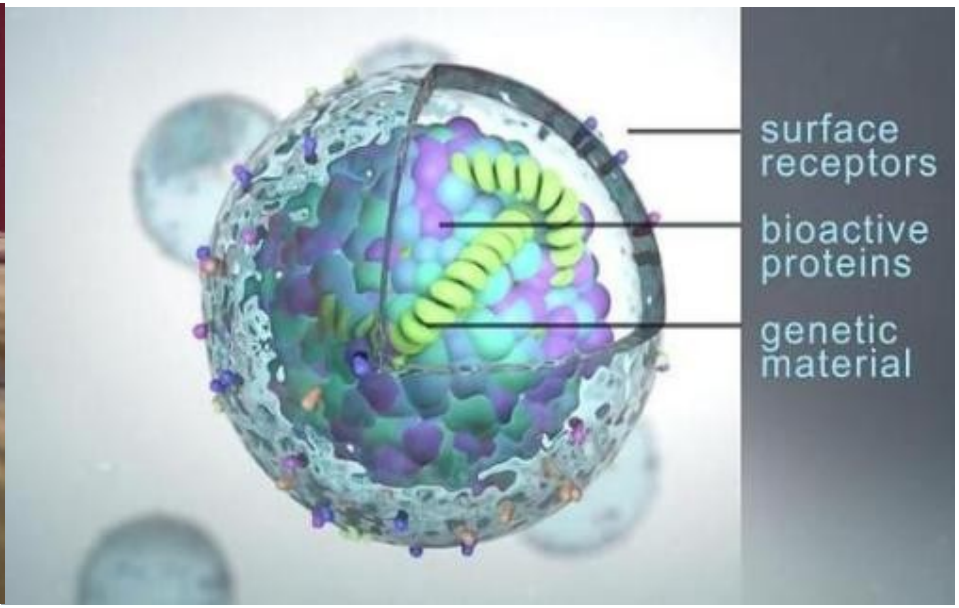
- **Allogeneic therapy is particularly beneficial in CLI:**
  - Consistent quality of cell lines
  - Cells are ready when patient needs them

** Katare et al, Arteriosclerosis, Thrombosis and Vascular Biology, 2014*

Exosome nanomedicine platform

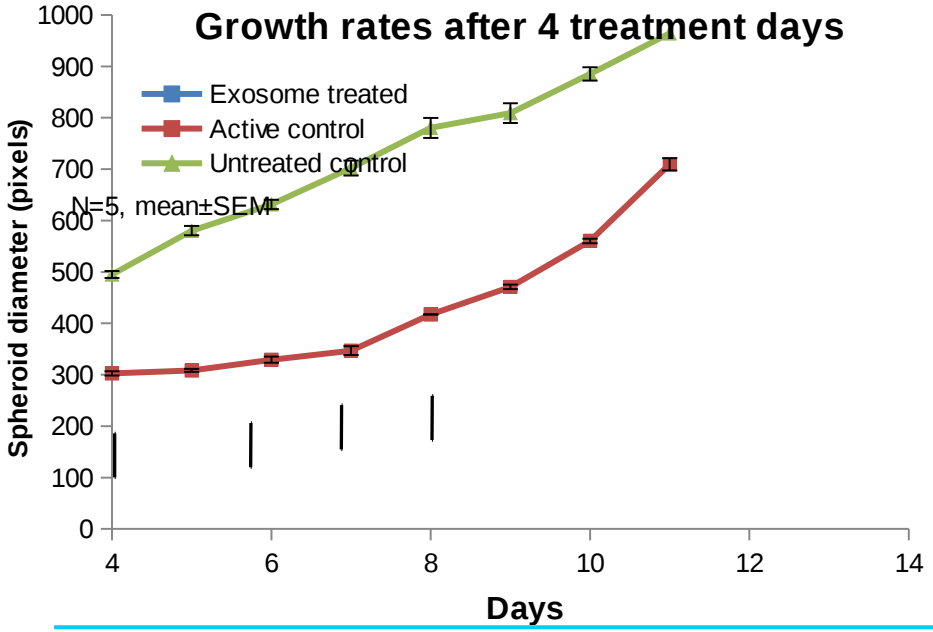
- Stem cell derived exosomes: potential new nanomedicine (cancer)
- Exosomes are nano-sized vesicles that are important in cell to cell signalling
- Multiple patent applications filed on CTX-derived exosomes

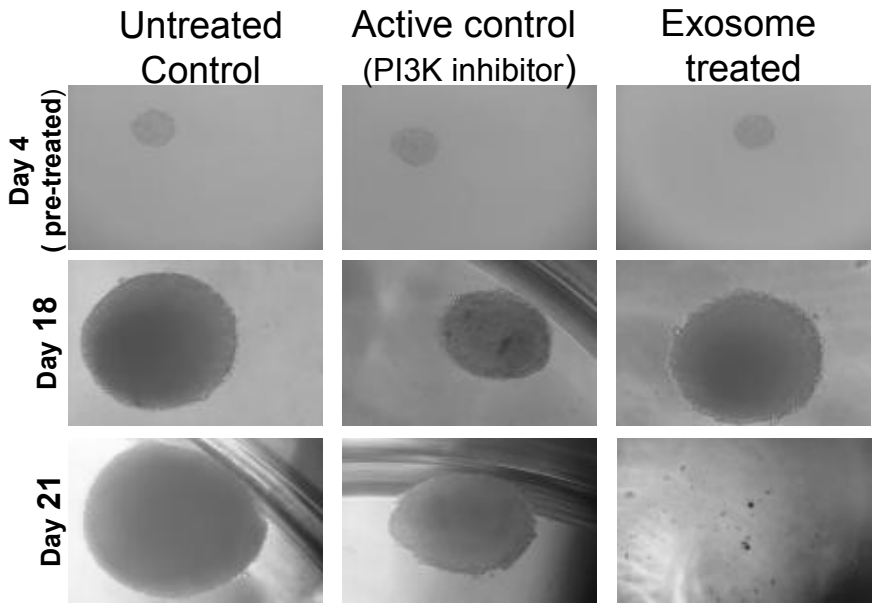




Exosome data* and development

- Exosomes inhibit glioblastoma cell migration
- Exosomes induce *in vitro* derived glioblastoma spheroids to break-up after 17-21 days in culture
- Similar results seen in early pre-clinical animal studies
- First-in-man clinical study planned for 2017

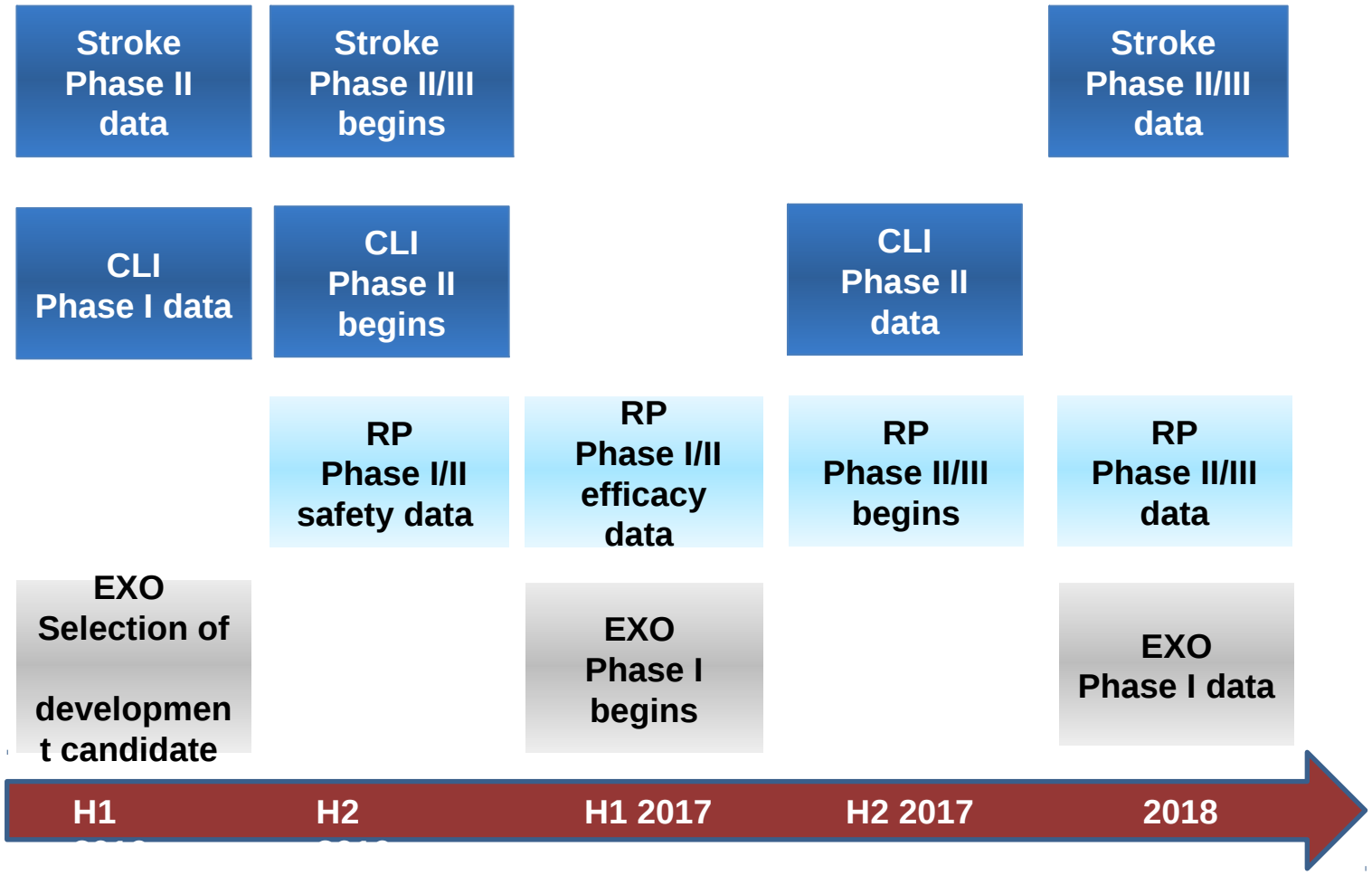




- * *unpublished*

Projected *funded* milesto

CTX programmes
hRPC programme
Exosome programme



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Senior Management and Board of Directors

Senior Management

Olav Hellebø	Chief Executive Officer (Schering-Plough, Novartis, UCB, Clavis)
Michael Hunt ACA	Chief Financial Officer (Biocompatibles, Bunzl)
Dr. John Sinden	Chief Scientific Officer and co-Founder
Dr. Randolph Corteling	Head of Research
Sharon Grimster	VP Development and General Manager, Wales (F-Star, Antisoma, Celltech)
Dr. Julian Howell	Chief Medical Officer (Shield, ProStrakan, Roche, Pharmion)
Shaun Stapleton	Head of Regulatory Affairs (Ely Lilly, Boehringer Ingelheim, Ipsen, RRG)

Non-executive Board

John Berriman	Chairman (Algeta, Heptares, Abingworth)
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Dr. Tim Corn	(Jazz Pharma, EUSA Pharma, Circassia, Glaxo)
Prof Sir Chris Evans	(Arthurian, Excalibur)
Dr. Paul Harper	(Physiomics, Sareum, CAT, Glaxo)
Dr Michael Owen	Chair – ReNeuron Scientific Advisory Board (Zealand Pharma, Avacta, Kymab, GSK, ICRF)

Investment proposition

- **Differentiated** stem cell technology platforms – **scalable & cost effective**
- Strong competitive position – first mover, with **very high barriers to entry**
- **Significant disease targets**: blockbuster potential in stroke disability and CLI
- Building a **high-value clinical portfolio** - 3 Phase II trials in progress or completed across 2 platforms within the next 12 months
- **Well backed and well funded** – strong institutional shareholder base
- Strong leadership team
- **Significant opportunity for further value build**, driven by clinical data and subsequent commercial development deals

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