



# Corporate presentation Innovators & Investors Forum 3 February 2015

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#### **ReNeuron Overview**

- A leading player globally in the high-growth field of stem cell therapeutics
- Founded 1998 quoted on AIM (RENE.L). Market cap approx £60m
- Addressing high-value unmet medical needs significant market opportunities
- At the forefront in stroke disability promising clinical data
- Very strong underlying stem cell technology platforms
- Experienced management team supported by key opinion-leading clinical advisers
- Strong patents
- Significant clinical milestones targeted over next 24 months



#### Well-backed and well-funded

Principal Institutional Investors	Holding
Woodford Investment Management	20.0%
Wales Life Science Fund	11.2%
Abingworth	5.3%
Invesco	4.9%
Majedie	4.6%

- Raised £25.4 million of equity funding in July 2013
- Received £11.8 million in grant commitments in 2013
- Funded to achieve key value inflection points within the next two years



#### **Board of Directors**

#### Management

Olav Hellebo Chief Executive Officer (Clavis, UCB, Novartis, Schering-Plough)

Michael Hunt ACA Chief Financial Officer (Biocompatibles, Bunzl)

**Dr. John Sinden** Chief Scientific Officer and co-Founder

#### **Non-Executive Board**

Bryan Morton Chairman (EUSA Pharma, Zeneus, Dechra, Oxford Biotherapeutics, BMS, Merck)

**John Berriman** (Algeta, Heptares, Abingworth)

Simon Cartmell (ApaTech, Celltech, Glaxo)

Dr. Tim Corn (EUSA Pharma, Circassia, Glaxo)

Mark Docherty ACA (Excalibur)

**Prof Sir Chris Evans** (Arthurian, Excalibur)

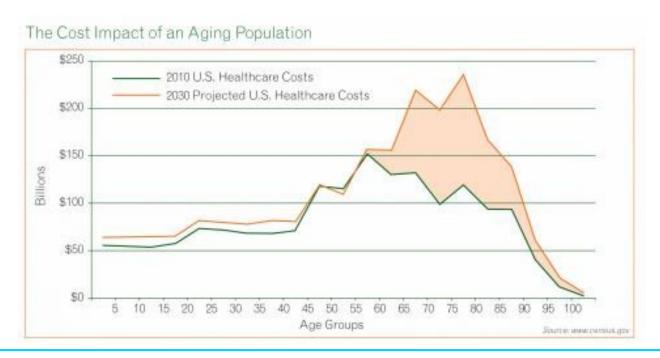
**Dr. Paul Harper** (Physiomics, Sareum, CAT, Glaxo)



# Why is Regenerative Medicine so important?

#### "WE ARE ON THE BRINK OF A NEW ERA OF MEDICINE

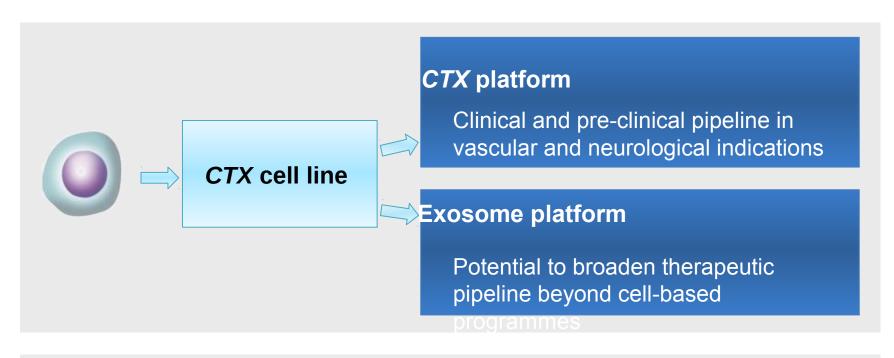
Regenerative medicine represents a new paradigm in human health, with the potential to resolve unmet medical needs by addressing the underlying causes of disease."

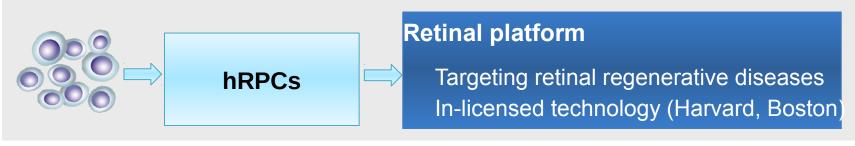






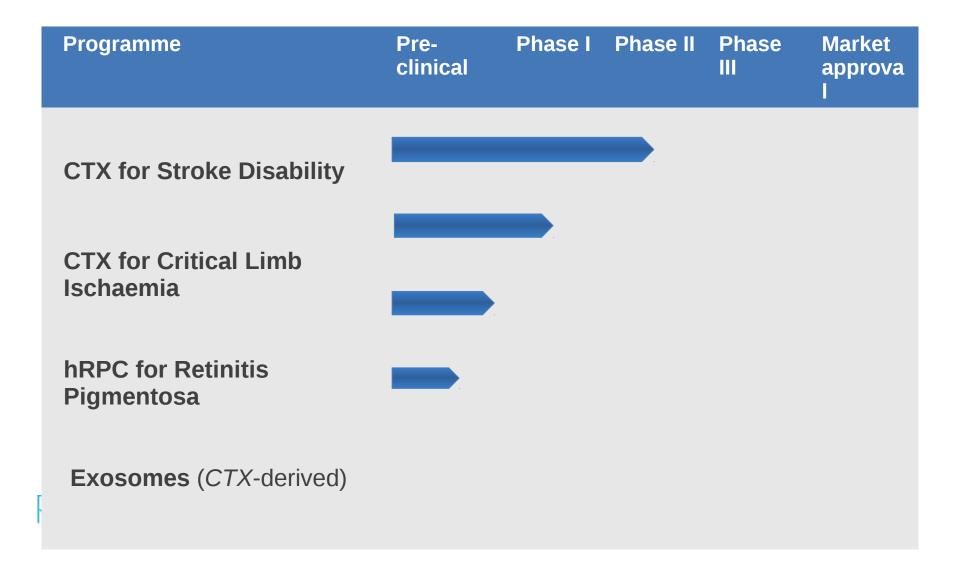
### Unique stem cell technology platforms







# Therapeutic pipeline



### **Targeting stroke disability**

- Stroke is the single largest cause of adult disability in the developed world
- Approx 800,000 people suffer a stroke in the US each year
- Annual healthcare/social care costs of stroke survivors: >\$70 billion in the US
- No pharmaceutical treatment options available beyond 4 hours
- Blockbuster market opportunity (multi-\$billion)
- Pre-clinical efficacy of our CTX cells in stroke demonstrated in multiple studies published in peer-reviewed scientific journals
- Target is to improve recovery of motor function in disabled stroke survivors



# Clinical development programme for stroke

- Phase I clinical trial completed in 11 disabled ischaemic stroke patients
- No cell-related or immunological adverse events
- Evidence of sustained reductions in neurological impairment and spasticity
- Phase II clinical trial to evaluate efficacy underway in the UK – first read-out in late 2015





# Targeting Critical Limb Ischaemia (CLI)

- Loss of blood flow to lower limb, leading to gangrene, amputation and death
- Common in diabetics
  - 160,000 legs amputated each year in the US due to CLI
  - Lifetime cost >\$0.5 million for each amputation
  - No current treatment options except surgery (20-50% ineligible for this)
  - Blockbuster market opportunity (multi-\$billion)
  - Published pre-clinical efficacy data demonstrating that CTX cells restore blood flow
  - Phase I clinical trial underway in UK Phase II clinical trial planned to commence later in 2015

- Target is to improve blood flow, leading to reduction in
  - incidence of death or amputation



## **Targeting Retinitis Pigmentosa (RP)**

- RP is an inherited, degenerative eye disease
- Causes severe vision impairment and often blindness
- ReNeuron's treatment for RP has Orphan Drug Designation in EU and the US (market exclusivity advantages)
- Published pre-clinical data demonstrating that our hRPC cells improve visual acuity

Phase I/II clinical trial scheduled

Normal vision



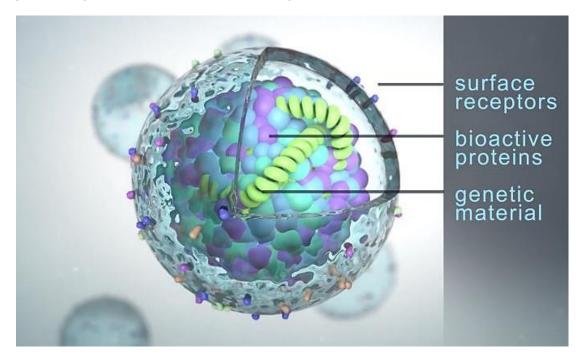
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RP vision



### Exosomes – a new therapeutic platform

- Exosomes are nano-sized vesicles secreted by stem cells
- Thought to play a fundamental role in cell-to-cell signalling
- Exosomes can be readily harvested from CTX cell expansion process
- Therapeutic potential in tissue repair, fibrosis and cancer





#### CTX programmes **NRPC** programme

# Expected clinical milesto (retinal) Exosome programme

**Stroke** Stroke **Stroke** Phase II Phase II Phase III begins data begins **CLI Phase I** CLI Phase I **CLI Phase II CLI Phase II** begins data begins data Completion Phase I/II Phase I/II of pre-Phase I/II **IND** filed data clinical begins data (safety) (efficacy) studies Choice of 1st clinical initial trial begins indication **H2** H1 H1 2016 H<sub>2</sub> 2016 H2 2015



### **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 across 2 platforms in the next 12 months
- Well backed and well funded strong institutional shareholder base
- Strong leadership team
- Funded to reach key clinical milestones within the next 24 months, providing significant opportunity for value build/subsequent commercial development deals





#### Thank you!



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