

ReNeuron

Shareholder Presentation

AGM Trading update
September 2017



Changing
patients' lives

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Company overview



Global leader in allogeneic cell-based therapeutics



Unique platform technologies



Breadth of pipeline



Well backed and well funded



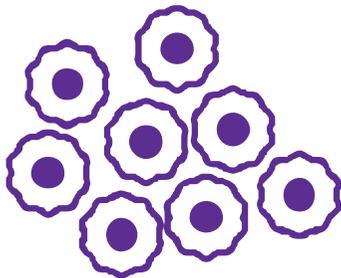
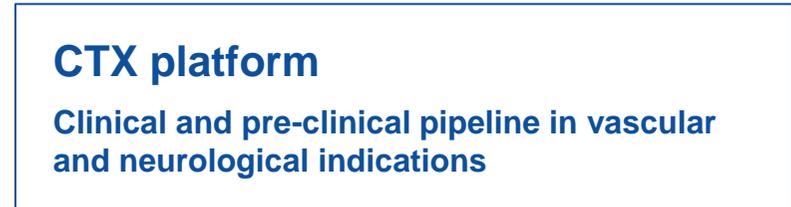
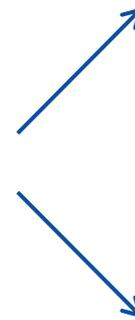
Strong management team



Focus on high value indications

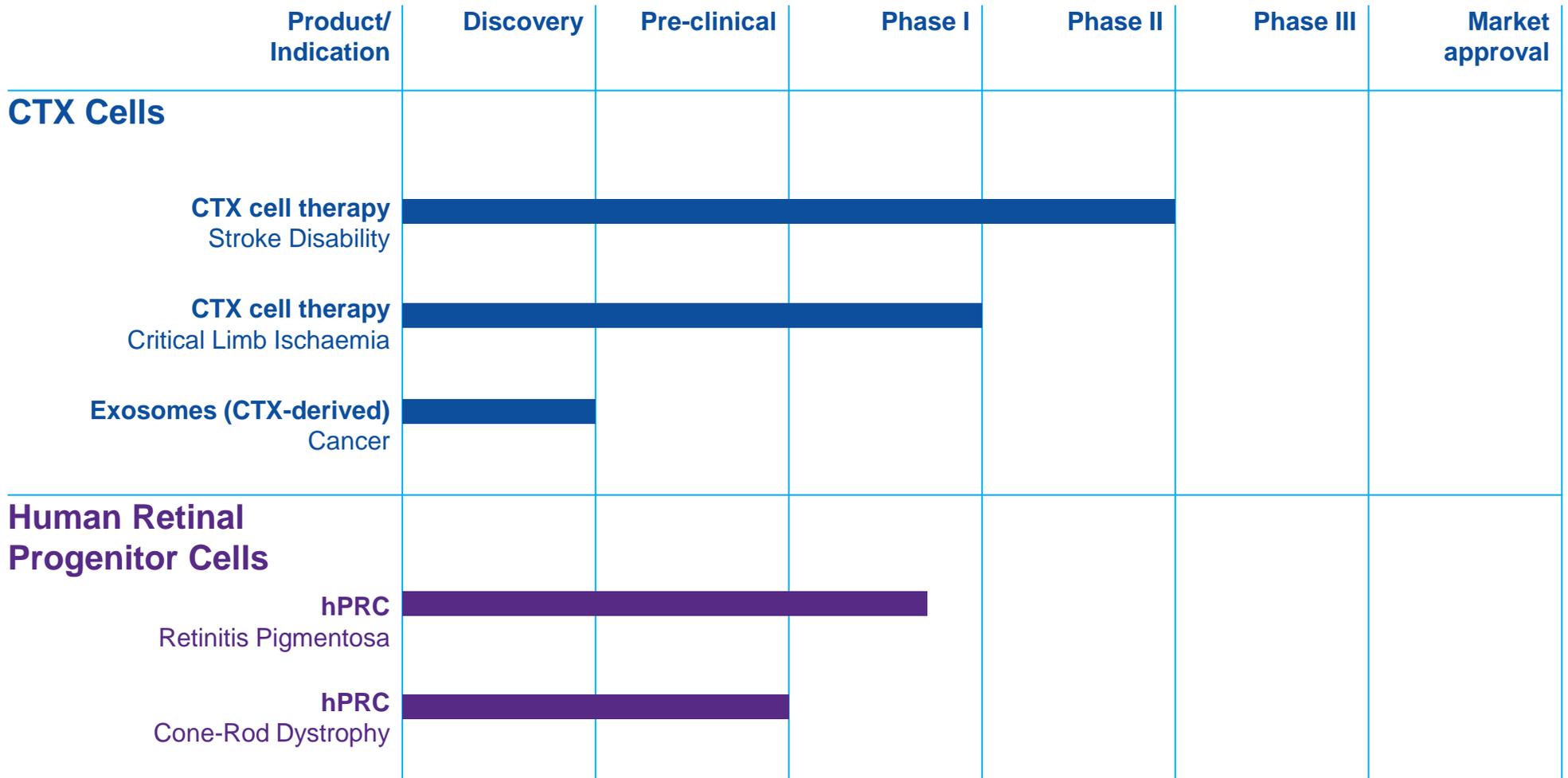


Unique platform technologies





Breadth of the pipeline





Well backed and well funded

Backed by major generalist and specialist life science institutional investors:

35.5%

Woodford Investment
Management

9.5%

Wales Life
Science Fund

9.3%

Invesco

5.7%

Aviva

£53 million

(\$66 million)

Cash on balance sheet (as at 31 March 2017)



Focused strategy helping patients without any treatment options

1

Develop best-in-class cell based therapies for life changing high value products

2

Gain clinical validation for our therapeutic programmes via robust clinical trials in well regulated territories.

3

Realise value for our technologies and therapeutic programmes via direct sales or substantial licence deals

Market potential according to analyst estimates*

Indication	Assumptions to 2026	Peak Sales (\$bn)
CTX for stroke	1.76 million strokes/year (total US/EU/Japan) 85% survival, 85 % ischaemic Peak penetration 5% US/EU/Japan Treatment cost \$40,000 EU to \$60,000 US/Japan	1.1 - 3.9
hRPC for RP	Prevalence 1:4000, ~244,000 cases (total US/EU/Japan) Peak penetration 7.5% US/ EU Per-eye treatment cost \$50,000 EU to \$75,000 US/Japan	0.5 - 1.8

*Stifel July 2016, N+1 Singer April 2017, Edison May 2017

- Applicability of hRPCs in other hard-to-treat ophthalmic diseases could provide upside potential
- Longer-term upside from exosome platform

CTX for stroke disability: unmet medical need



- Stroke is the single largest cause of adult disability
- Annual health/social costs: >\$70 billion in the US
- Only one pharmaceutical treatment option available within 4 hours of stroke onset
- No treatment options available for stroke patients months to years later
- CTX administration promotes repair in the damaged brain

PISCES II stroke clinical trial - conclusions

- Rate of patient improvement in patients with established disability due to stroke has greatly exceeded what we expected
 - 15 of 21 patients responded to one or more of the four efficacy measures
 - Response rate on mRS was greater than expected – use as primary measure in future studies
- Patients ready to enter study from 6 months post-stroke
- CTX intracerebral injection was well tolerated
 - Adverse Events were attributed to surgical procedure or stroke complications
 - 1 death due to sepsis, 7 months after CTX treatment. Assessed as not attributable to treatment.
- 33% response rate in mRS outcome measure
- 53% response rate in evaluable patients for BI measure



Potential of CTX in stroke warrants moving into a Phase III study

Phase III study - PISCES III



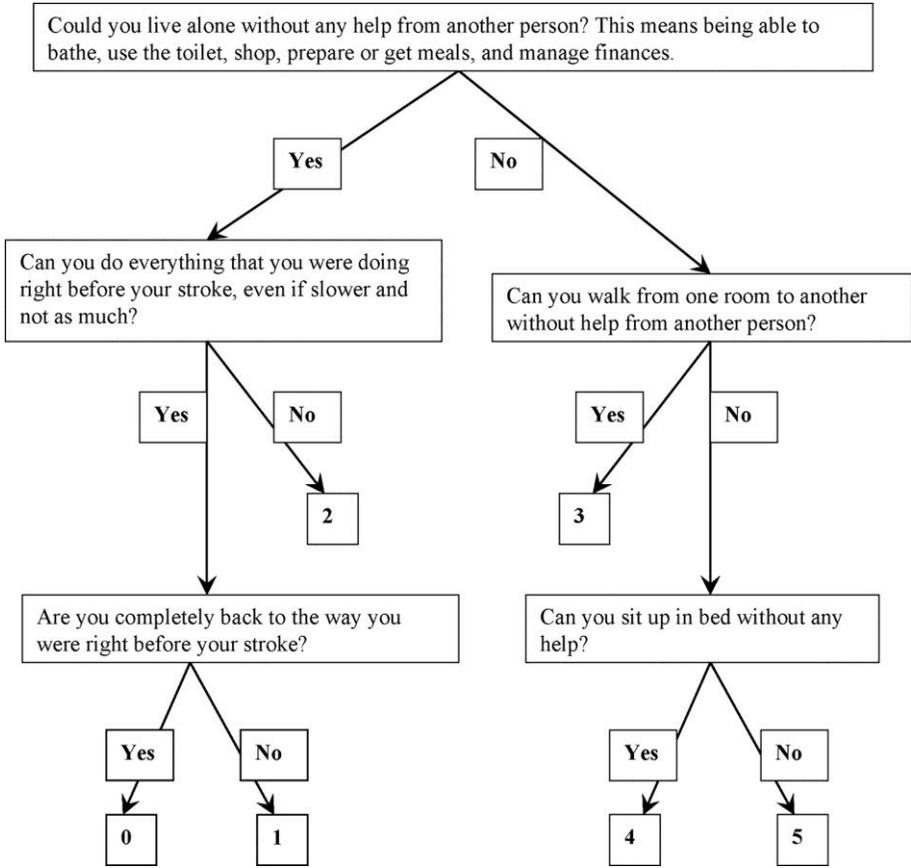
PHASE III
INVESTIGATION
OF STEM CELLS
IN STROKE

- Randomised, controlled study with placebo surgery
- Entry criteria: Ischemic stroke 6-12 months prior and modified Rankin Score (mRS) of 3 or 4
- Primary endpoint: Response as measured by mRS six months post treatment
- US and European sites use “Hub and Spoke” – reduced number of surgical sites
 - Improves blinding by eliminating interaction of surgical staff and assessors
- 220 patients, 1 to 1 randomisation, CTX 20 million cell dose as used in PISCES II
- Discussions held with FDA / EMA, received favourable responses
- Commencing in early 2018 – Data expected early 2020
- Expected response rate in mRS is clinically meaningful and commercially viable
 - Enrolling patients with some shoulder movement
 - Designed to show statistical significance with a treatment efficacy rate of 35% and placebo response of 15%
 - Alteplase had 13% increase in favourable outcome in pivotal study for acute stroke (n=333)

Discussions held with FDA on pivotal study design

Modified Rankin Score

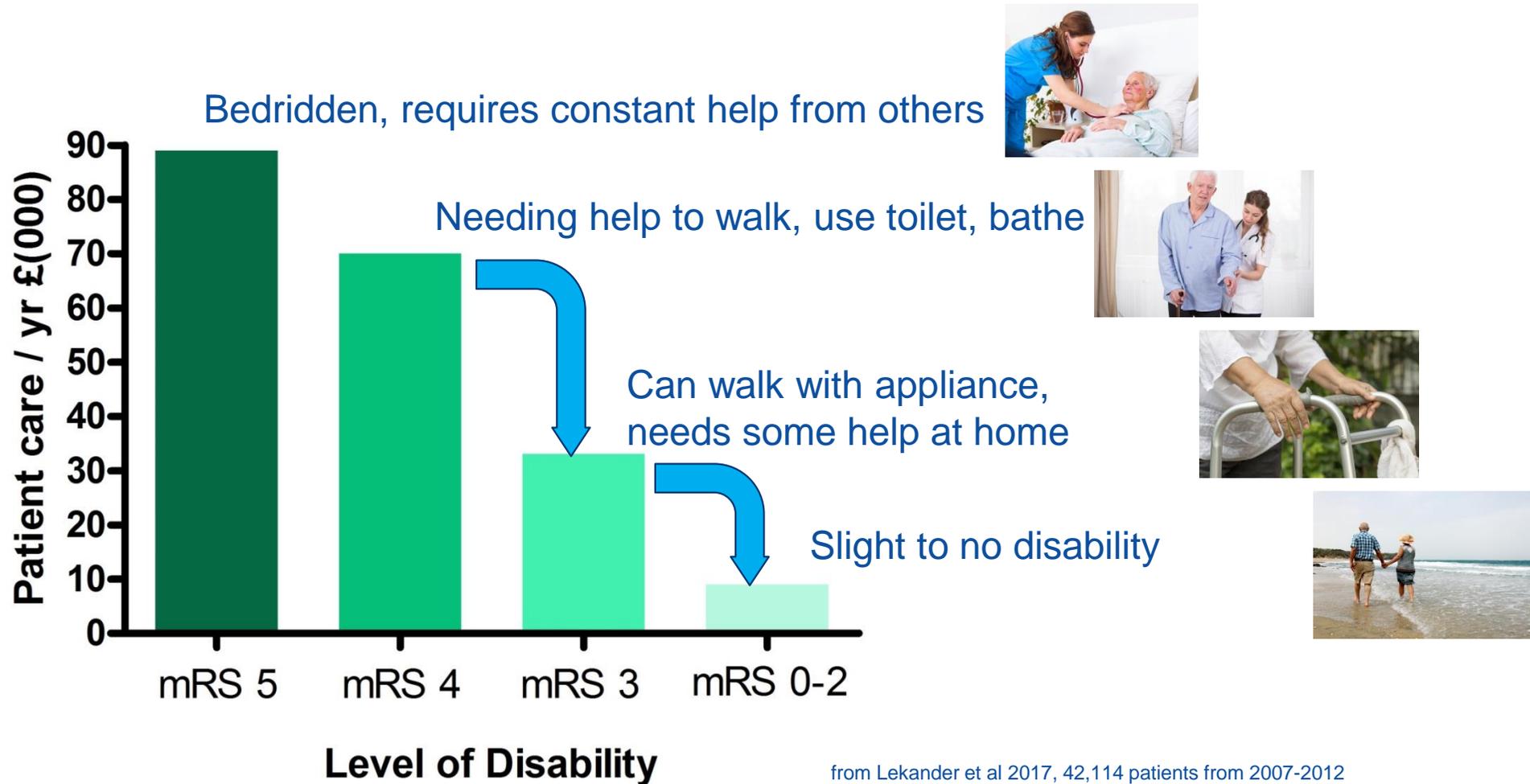
Category
5 Bedridden, completely dependent on others
4 Needing help to walk, use toilet, bathe
3 Can walk, but need still need help at home
2 Mostly recovered, but still has limitations
1 Slower than before, but no limitations
0 Back to pre-stroke life



Algorithm from Bruno et al, 2010

Improvement by one category is a significant change in a patient's life

Costs of disability – mRS scale



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

Improvements in disability result in substantial reductions in patient care costs

Retinal platform

- The eye does not regenerate lost cells
- Small changes in the retina will have a great impact in vision
- Programme based on human retinal progenitor cells (hRPCs)
 - Preclinical testing programme demonstrated:
 - Rescue of photoreceptors to preserve vision
 - Maturation of injected hRPCs
 - Approval of hRPC frozen formulation
 - Ship and thaw on demand
- Collaborations:
 - Schepens Eye Research Institute (Harvard Medical School)
- Targeting Retinitis pigmentosa, Cone rod dystrophy



Broad application across a range of retinal diseases

Retinitis pigmentosa

- RP is an inherited, degenerative eye disease
 - Onset from 20s to middle age
 - Initial damage in outer retina
 - Results in tunnel vision then 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 ongoing in the US
 - Phase I/II readouts in Q4 2017 & H2 2018 with further Phase II efficacy data in larger patient cohort in mid-2019



RP vision

There is no approved drug treatment for Retinitis pigmentosa

Cone rod dystrophy

- CRD is an inherited, degenerative eye disease
 - First noticed in childhood
 - Initial damage to cones in central retina
 - Loss of visual acuity and colour vision
 - Progresses to blindness
 - Incidence of CRD is 1:40,000 in the US
- Second therapeutic target for hRPCs
- Use safety data from RP Phase I study
- Conduct Phase II trial alongside RP study
 - Commence in H1 2018
 - Readout in H2 2019



CRD vision

image from MD Support

Exosome nanomedicine development

- Exosome therapeutic candidate selected (ExoPr0)
- Pre-clinical data
 - ExoPr0 inhibits glioblastoma cell migration
 - ExoPr0 reduces tumour volume in a CDX (cell-line derived xenograft) mouse model of glioblastoma
 - Published data* identifies micro-RNAs contained within ExoPr0 responsible for regulating cell growth and apoptosis in cancer
 - ExoPr0 crosses blood brain barrier allowing treatment of a number of conditions
- Development of platform technology for the delivery of therapeutic silencing RNA
 - Target genes over-expressed in cancer indications
- £2.1m Innovate UK grant awarded to pursue ExoPr0 pre-clinical development
 - Collaborators – Netherlands Cancer Institute, UCL, Cell & Gene Therapy Catapult

ReNeuron is a leader in a new field of medicine

* Stevanato et al. PLOS ONE (2016)

Key clinical milestones by programme

CTX for stroke disability

- H2 2017 - Phase II 12 month follow-up data
- H1 2018 - Phase III commencement
- H1 2020 - Phase III data

hRPC for retinitis pigmentosa

- H2 2017 - Phase I/II short-term data
- H2 2018 - Phase I/II longer-term data
- Mid-2019 - Phase II data (enlarged patient group)*

hRPC for cone-rod dystrophy

- H1 2018 - Phase II commencement
- H2 2019 - Phase II data*

Exosomes for cancer

- H2 2018/H1 2019 - Phase I commencement

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