EDISON

StemCells

Trial update

Phase II trials underway

Pharma & biotech

StemCells has announced transplantation of the first patient in a recently initiated Phase II trial, evaluating its human central nervous system stem cells (HuCNS-SC) as a treatment for dry age-related macular degeneration (AMD). Progression into the Phase II RADIANT trial was supported by the top-line results of its Phase I/II dose-ranging trial in dry AMD announced in June. Additionally, the company began dosing the first cohort in its Phase II PATHWAY study in cervical spinal cord injuries (CSI) earlier this year. At a price of $0.41, the shares offer considerable potential, currently trading significantly below our risk-adjusted fair value of $1.82 per share.

Year end	Revenue ($m)	PBT* ($m)	EPS* ($)	DPS ($)	P/E (x)	Yield (%)
12/13	1.2	(29.7)	(0.68)	0.0	N/A	N/A
12/14	1.0	(32.2)	(0.56)	0.0	N/A	N/A
12/15e	0.2	(34.1)	(0.35)	0.0	N/A	N/A
12/16e	0.2	(35.2)	(0.33)	0.0	N/A	N/A

Note: *PBT and EPS are normalized, excluding intangible amortization, exceptional items and share-based payments.

Transplantation of first patient in Phase II in dry AMD

Final results of the 15-patient Phase I/II AMD trial investigating safety and efficacy of sub-retinal transplantation of HuCNS-SC were presented on 26 June at the Annual Meeting of the International Society for Stem Cell Research (ISSCR) in Stockholm, supporting progression into Phase II. The trial met its primary endpoint showing favorable safety and tolerability, also showing improvements in visual function as measured by best-corrected visual acuity (BCVA) and contrast sensitivity. However, the progression of geographic atrophy (GA) was obfuscated by complex and subjective readouts in a largely unrepresentative patient population. The recently initiated Phase II study will encompass a more homogenous study population with patients with less advanced disease (and more measurable GA).

Patent infringement case reaches resolution

StemCells' share price has also been negatively affected by the recently announced dismissal of its patent infringement case against Neuralstem by the US Federal District Court of Maryland. StemCells had hoped to gain sole ownership over six patents related to its human neural stem cells. While disappointing for the company, the decision should have little direct impact on its ongoing clinical trial programs.

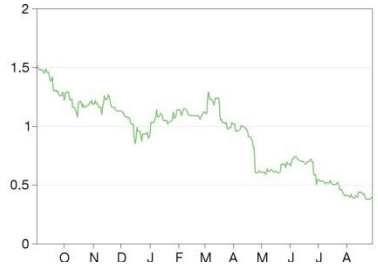
Valuation: $198m from $205m

On 6 August, StemCells reported Q215 results, which were generally in line with our expectations. Our DCF-based valuation of $198m ($1.82 per share) is adjusted only slightly from $205m ($1.95 per share) mainly on cash usage over the quarter. We do not include potential share dilution from current out-of-the-money warrants and restricted stock. The company's cash runway should enable operations to be funded into 2016.

1 September 2015

Price	**$0.41**
Market cap	**$44m**
Net cash ($m) at 30 June 2015	26.4
Shares in issue	108.5m
Free float	93%
Code	STEM
Primary exchange	NASDAQ
Secondary exchange	N/A

Share price performance



%	1m	3m	12m
Abs	(0.2)	(39.7)	(73.4)
Rel (local)	6.4	(35.6)	(73.0)
52-week high/low		$1.54	$0.38

Business description

StemCells is a US biotech company developing stem cell-based therapeutics. The lead clinical programme is based on HuCNS-SC (human neural stem cells) for spinal cord injury (SCI) and dry age-related macular degeneration (AMD), both in Phase II trials.

Next events

Interim six-month data PATHWAY study (cohort 1) in cervical SCI	Q415
Final Phase II PATHWAY results (cervical SCI)	2017
Final Phase II RADIANT results (dry AMD)	2017

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Edison profile page

Two Phase II trials in areas with major unmet need

StemCells is steadily progressing its pipeline of proprietary human central nervous system stem cells (HuCNS-SC), focusing on CNS disorders. Trials in its two lead indications, spinal cord injuries (SCI) and dry age-related macular degeneration (AMD) have now moved from early and encouraging dose-finding and initial efficacy to larger Phase II studies powered for proof of concept. Both studies are expected to deliver full and final results in 2017.

- Enrolment has started in a Phase II study in GA of age-related AMD. The study was supported by final results presented in June of a Phase I/II study showing good safety tolerability with promising efficacy in a subpopulation of the patient population with a less advanced stage of the disease (and targeted in the Phase II design).
- The ongoing Phase II PATHWAY trial in spinal cord injury has been enrolling its second cohort in the study from early June, while Health Canada has provided approval for an additional site in Canada, adding to the existing eight sites in the US. Interim results are expected in Q415.

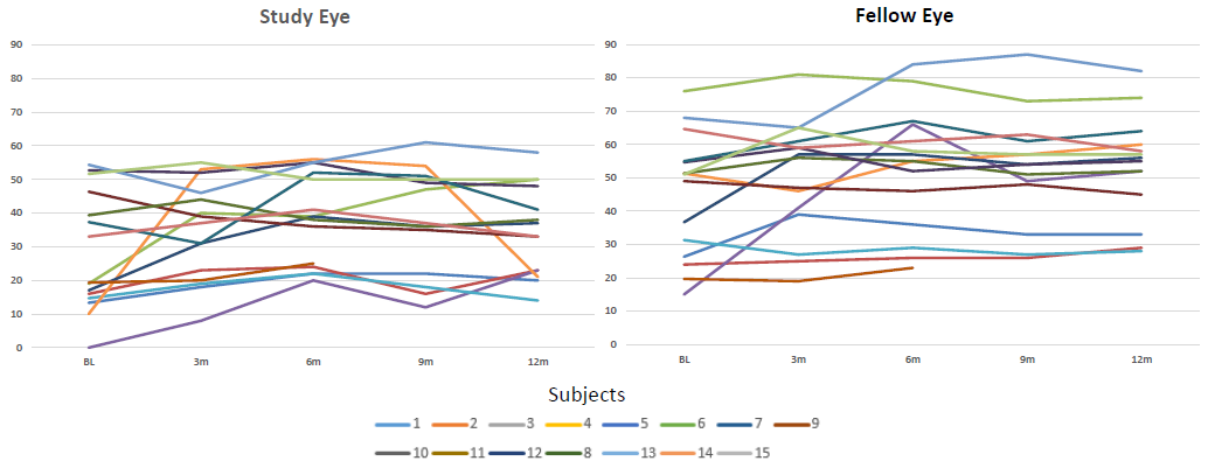
Progression in dry AMD

Phase I/II presented at ISSCR

Final results of the 15-patient Phase I/II AMD trial investigating safety and efficacy of subretinal transplantation of HuCNS-SC were presented on 26 June at the Annual Meeting of the ISSCR in Stockholm. The trial was initiated on the basis of significant indicative preclinical efficacy, as evidenced in an in vivo rat model of AMD, showing that implantation of the HuCNS-SC cells could protect the at-risk cells of the macula from death and prevent or slow down vision loss. The multi-center, open-label, dose-escalation Phase I/II trial consisted of three cohorts, each receiving a different number of cells: Group 1a received 200,000 cells and Group 1b and 2 were given 1m cells. The primary objective was safety including assessment of visual function and disease status by direct retinal examination. The secondary objective, preliminary efficacy, included tests for BCVA, optical coherence tomography (OCT) and fundus autofluorescence (FAF) to measure underlying GA.

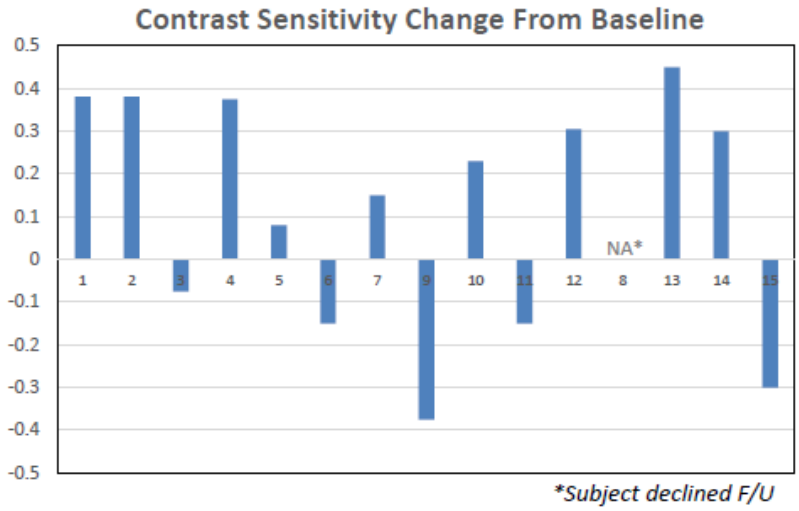
The interim and preliminary final study analysis of the Phase I/II trial showed improvements in terms of visual function, as measured by BCVA and contrast sensitivity. Gains in contrast sensitivity (a measure that helps people drive at night or gauge the depth of a poorly lit staircase) were seen in the majority of subjects with over half of the participants showing an improvement of two or more three letter group gains in contrast sensitivity based on the scoring template. Other anatomic assessments were encouraging where increased macular volume and foveal thickness were observed in the study vs fellow eye. The interim and final results also confirmed favorable safety and tolerability.

Exhibit 1: Phase I/II AMD – BCVA at 12 months*



Source: StemCells, ISSCR, 26 June 2015. Note: Vertical axis is growth in lesion size mm^2.

Exhibit 2: Contrast sensitivity from baseline at 12 months*



Source: StemCells, ISSCR, 26 June 2015. Note: Vertical axis shows change in 3 letter group score (each three letter group = 0.15 improvement).

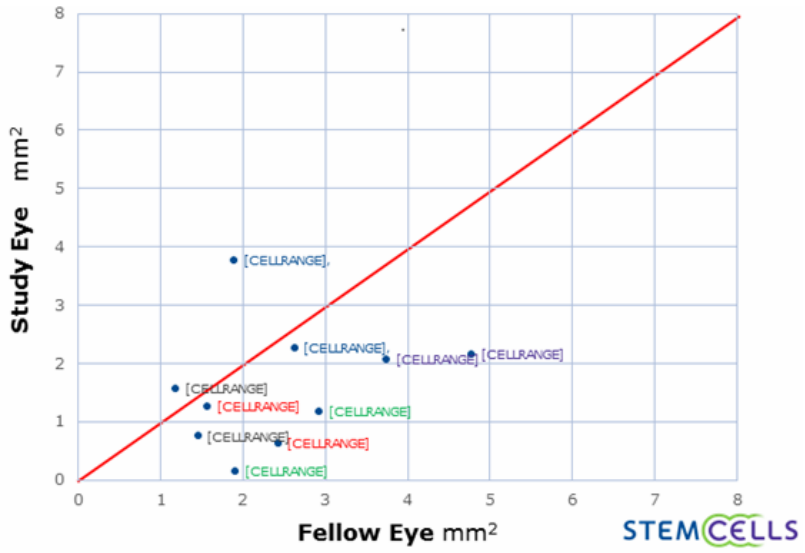
In terms of GA, the interim efficacy results presented in June 2014 were encouraging, and showed a 70% reduction in the rate of GA compared to the control eye (untreated eye of same patient) and a 65% reduction in the rate of GA compared to the expected natural history of the disease following a single 200,000 cell dose. This is especially important as the goal of this therapy is preservation. Even at the lower cell dose, four of the eight patients with 12-months' follow-up (M12) had GA growth rates in the treated eye notably lower than the control eye.

Following the conclusion of the study, two independent analyses of GA (retinal pigment epithelium loss) were conducted for all cohorts, as measured by FAF. A prospective analysis showed an overall strong trend for decreased GA progression in the study eye vs the fellow eye, consistent with the findings in the interim evaluation. However, a further post-hoc analysis did not reveal a similar trend with greater than anticipated variability in the grading of the images. We point out that these analyses are hampered by the open-label nature of the trial, as well as the small sample size, while the high learning capability on tests also confounds data. It is therefore difficult to draw conclusions from the early data.

StemCells subsequently presented further evaluation of the final Phase I/II data in its Q2 earnings call. At that time management emphasized that criteria for the patient population of the Phase I/II

study did not preclude patients with severe and/or advanced disease – patients that would not be included in the subsequent Phase II study focused on efficacy. The study comprised a diverse patient group, which included those with assorted GA lesions and morphology impeding the results. Disparity in the analyses was thought to be mainly on account of measuring the large and complex lesions presented by 10 of the 15 patients in the study, none of which would have met the criteria for the ongoing Phase II efficacy study. Lesions in the patients with advanced disease progression tended to be large and difficult to analyze objectively, particularly given rugged edges of the lesions and in some cases 'island lesions' off the main shaded area. However, favorable data from the five patients with less advanced GA (and which would fit the Phase II criteria) are shown in Exhibit 3 and support the Phase II trial design. The chart demonstrates that the majority of patients had lower growth of lesions in the study vs the fellow eye. Patients will be followed for a further four years as part of a long-term follow-up trial. Overall, the Phase I/II results provided the support for progression into the recently initiated Phase II study, which encompasses a more homogenous study population with patients with less advanced disease (and more measurable GA).

Exhibit 3: Phase I/II AMD Trial – Phase II eligible patients



Source: StemCells Q215 earnings presentation. Note: Reading Center and site GA assessment by FAF change from BL over 12m.

Phase II RADIANT trial initiated in dry AMD with first transplantation

On 20 July, StemCells announced the start of a Phase II trial, RADIANT in GA, the most advanced form of age-related macular degeneration (dry AMD). Transplantation of the company's human neural stem cells was completed at the Retina Foundation of the Southwest for the first patient in the study. The Phase II proof-of-concept study, enrolling 63 patients, is to evaluate both safety and efficacy of the treatments in intervals over 12 months, assessing both anatomic and functional changes. Patients, all suffering bilateral GA-AMD (geographic atrophy associated with age-related AMD in both eyes) will receive subretinal injection of HuCNS-SC cells into the eye with the inferior BCVA. The study aims to show a reduction in disease progression in the treated vs the control or fellow eye, which will remain untreated. During the Q215 results conference call, management spoke of the strong interest by the medical community in the study, for which full enrolment is expected in 2016. Initial top-line data from the trial are expected in mid-2017.

Age-related macular degeneration is a condition that generally affects people over 50 years of age, and increases in prevalence with age. An estimated 18 million people suffer from some form of AMD in the US and the disease is the number one cause of severe vision loss and legal blindness in those over the age of 55. There are two forms of AMD, dry and wet, with all AMD beginning as the dry form, and approximately 10-15% of cases progressing to the wet form. There are a number

of treatments for wet AMD, including anti-VEGF agents such as Lucentis (worldwide FY14 sales $4bn by Novartis/Roche) and Eylea (WW FY14 sales $2.8bn by Bayer/Regeneron). There are no approved treatments for dry AMD, which points to the considerable opportunity for an early-entrant treatment. The use of stem cells for ophthalmic indications has generated significant interest owing to the potential restorative power of these cells in a localized and protected environment. There are only a handful of stem cell treatments currently under investigation for dry AMD, of which none is neural, and therefore would likely be complementary to StemCell's implantation therapy.

SCI PATHWAY study approved in Canada

Earlier this year, StemCells commenced dosing of the first cohort of patients in its Phase II 52-patient PATHWAY study in cervical SCI. Safety and early signs of efficacy have been established in thoracic SC in a Phase I/II trial and top-line 12-month results presented at the joint Spinal Cord Society (ISCoS) and American Spinal Injury Association (ASIA) meeting. In early June the scope of the study was expanded, with authorization by Health Canada to expand PATHWAY into Canada, in addition to the eight sites currently actively recruiting in the US. Interim six-month data in the open-label, dose-escalation arm are expected in the first cohort of seven patients by the end of 2015 and will focus on change in motor function in the upper extremities. Full results of all cohorts are anticipated in 2017. We forecast sales to peak at $290m in 2026 in spinal cord injury in the EU and the US.

Patent infringement case comes to a close

In late July StemCells announced that the longstanding patent infringement suit against Neuralstem on a series of patents, the Weiss and Reynolds patents by name, was dismissed with prejudice by the US Federal District Court of Maryland. The opinion was released on 22 July, ruling that a third-party scientist has been deemed to be co-owner and co-inventor of the six patents in the litigation. The news came as a disappointment to the company, which had hoped to claim full ownership for the patents. However, the suit has no bearing on the current operations of the company and its ongoing clinical trials. StemCells' IP portfolio includes multiple patent families, as well as proprietary expertise. Its CSI program, currently in Phase II trials, is well in advance of Neuralstem's, which is prioritizing the application of its stem cell technology in other disease areas. Neuralstem uses its neural stem technology primarily for treatment in CNS diseases with its lead clinical trial program in major depressive disorder (Phase II to be initiated in 2015), while Phase I trials are ongoing in schizophrenia, amyotrophic lateral sclerosis, CSI (chronic spinal cord injury) and ischemic stroke.

Valuation

Our valuation of StemCells is adjusted slightly to $198m ($1.82 per share) from $205m ($1.95 per share) primarily as a result of cash use over the quarter. We base our valuation on a sum-of-the-parts DCF applying our standard 12.5% discount rate. We do not include potential share dilution from current out-of-the-money warrants and restricted stock.

Exhibit 4: Valuation model and key assumptions

Product	Status	Launch	NPV ($m)	Peak sales ($m)	Probability of success	Royalty rate	rNPV ($m)	rNPV/ share ($)	Key assumptions
HuCNS-SC in AMD	Phase II	2021	670	2,050	25%	20%	160	1.48	US - GA annual incidence: 310,000; 15% severe vision loss; 25% peak penetration (2026); 1.5 eyes treated; $40,000/dose. EU - GA annual incidence: 680,000; 15% severe vision loss; 20% peak penetration (2026); 1.5 eyes treated; $32,000/dose.
HuCNS-SC in SCI	Phase II	2021	87	289	20%	20%	11	0.10	US - SCI annual incidence: 12,500; 25% peak penetration (2026); $50,000/dose. EU - SCI annual incidence: 10,500; 20% peak penetration (2026); $40,000/dose.
Portfolio total			757				171	1.58	
Net cash (end Q215)*							26.4	0.24	
Overall valuation							198	**1.82**	108.5m shares outstanding (undiluted)

Source: Edison Investment Research. Note: *Assuming completion CIRM loan forgiveness in full.

Financials

StemCells reported an operating loss at end June of $8.5m (vs $8.0m in Q214), which was generally in line with our expectations. Sales of $30,000 were reported in the quarter on royalties from licensing agreements. R&D costs moved up on the initiation of two Phase III efficacy trials (PATHWAY and RADIANT), as well as staff enhancements to strengthen clinical capabilities and product development. We continue to model R&D trending upward in the coming years as treatment candidates advance through development, but anticipate that pivotal Phase III trials will receive partial or full funding from potential partners. We forecast small and incremental increases in SG&A.

StemCells had $29.9m in cash as of 30 June 2015, up from $14.1m at the end of Q115, as it shored up its balance sheet through an equity raise earlier in the quarter yielding ~$25m net. The company's cash runway should enable operations to be funded into 2016. In its recent conference call, StemCells reiterated its intent to seek non-dilutive sources of capital as a primary source of funding. Current outstanding warrants remain a potential future source of cash although, at an exercise price of $0.42, are out-of-the-money (share price as of 19 August) and are not incorporated into our model.

Exhibit 5: Financial summary

$'000s	2012	2013	2014	2015e	2016e
Year-end 31 December	US GAAP	US GAAP	US GAAP	US GAAP	US GAAP
PROFIT & LOSS					
Revenue	1,368	1,203	1,012	171	200
Cost of Sales	(263)	(317)	0	0	0
Gross Profit	1,105	887	1,012	171	200
Research and development	(15,847)	(20,534)	(21,503)	(23,617)	(24,798)
General & administrative	(7,447)	(8,897)	(10,420)	(10,153)	(10,458)
EBITDA	(23,181)	(29,602)	(32,218)	(35,005)	(36,422)
Operating Profit (before GW and except.)	(22,189)	(28,544)	(30,910)	(33,599)	(35,056)
Intangible Amortization	0	0	0	0	0
Exceptionals/Other	(356)	(62)	0	0	0
Operating Profit	(22,546)	(28,605)	(30,910)	(33,599)	(35,056)
Net Interest	(35)	(1,155)	(1,287)	(480)	(163)
Other (includes change in fair value of warrants)	(5,911)	3,322	(544)	(206)	0
Profit Before Tax (norm)	(22,224)	(29,699)	(32,197)	(34,079)	(35,219)
Profit Before Tax (FRS 3)	(28,491)	(26,439)	(32,741)	(34,285)	(35,219)
Tax	0	0	0	0	0
Deferred tax	0	0	0	0	0
Profit After Tax (norm)	(22,224)	(29,699)	(32,197)	(34,079)	(35,219)
Profit After Tax (FRS 3)	(28,491)	(26,439)	(32,741)	(34,285)	(35,219)
Average Number of Shares Outstanding (m)	28.8	43.4	61.6	98.0	108.5
EPS - normalized (c)	(77.0)	(68.2)	(56.3)	(34.6)	(32.5)
EPS - FRS 3 (c)	(77.0)	(68.2)	(56.3)	(34.6)	(32.5)
Dividend per share (c)	0.0	0.0	0.0	0.0	0.0
BALANCE SHEET					
Fixed Assets	6,129	9,717	5,919	5,264	4,669
Intangible Assets	3,806	3,975	357	336	336
Tangible Assets	1,375	5,305	5,187	4,555	3,960
Other	947	437	375	373	373
Current Assets	24,041	31,840	26,508	14,689	20,329
Stocks	0	0	0	0	0
Debtors	110	109	159	17	17
Cash	22,372	30,585	24,988	13,456	19,096
Other	1,559	1,146	1,361	1,216	1,216
Current Liabilities	(5,097)	(9,132)	(11,498)	(7,858)	(5,113)
Creditors	(4,891)	(5,343)	(6,811)	(5,113)	(5,113)
Short term borrowings*	(206)	(3,789)	(4,686)	(2,745)	0
Long Term Liabilities	(11,089)	(17,471)	(15,059)	(5,468)	(45,468)
Long term borrowings**	(125)	(9,245)	(10,324)	(358)	(40,358)
Other long term liabilities	(10,964)	(8,226)	(4,734)	(5,110)	(5,110)
Net Assets	13,985	14,954	5,871	6,627	(25,583)
CASH FLOW					
Operating Cash Flow	(19,819)	(22,895)	(27,352)	(30,769)	(30,712)
Net Interest	(50)	(427)	0	(295)	(163)
Tax	0	0	0	0	0
Capex	(73)	(4,706)	(426)	(752)	(771)
Acquisitions/disposals	0	0	0	149	0
Financing	25,949	23,683	20,425	23,350	0
Dividends	0	0	0	0	0
Other	0	0	0	0	0
Net Cash Flow	6,006	(4,346)	(7,353)	(8,318)	(31,646)
Opening net debt/(cash)	(16,069)	(22,041)	(17,551)	(9,977)	(10,352)
HP finance leases initiated	0	0	0	0	0
Exchange rate movements	16	2	16	0	0
Other	(51)	(146)	(238)	8,693	31
Closing net debt/(cash)	(22,041)	(17,551)	(9,977)	(10,352)	21,263

Source: StemCells financial reports, Edison Investment Research. Note: *Silicon Valley Bank Loan ($3.5m outstanding as of 30 June 2015 fully payable by April 2016. **CIRM (California Institute for Regenerative Medicine) loan granted in April 2013: outstanding amount of $8.9m as of 30 June 2015 is expected to be forgiven in full, in accordance with the terms of the loan agreement (source: 10-Q, 30 June 2015).

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