EDISON

Factor Therapeutics

H117 results

Key venous leg ulcer Phase IIb underway

Pharma & biotech

Factor Therapeutics expects to report top-line results before the end of the year from its randomised Phase IIb trial of VF-001 in venous leg ulcer (VLU) patients. The trial has been designed to recruit patients with VLU with moderate severity, the subgroup that responded best to VF-001 in a previous Phase II trial. Positive results would enable submission for CE mark approval in Europe and clear the way to pivotal Phase III studies in the US. We roll our risked NPV model forward following release of H117 results, which lifts our valuation to A$108m or A$0.15 per share.

Year end	Revenue (A$m)	PBT* (A$m)	EPS* (c)	DPS* (c)	P/E (x)	Yield (%)
06/15	0.4	(7.1)	(4.0)	0.0	N/A	N/A
06/16	0.4	(4.1)	(3.0)	0.0	N/A	N/A
06/17e	2.6	(5.4)	(0.8)	0.0	N/A	N/A
06/18e	1.8	(4.6)	(0.6)	0.0	N/A	N/A

Note: *PBT and EPS are normalised, excluding amortisation of acquired intangibles, exceptional items and share-based payments.

Recruitment underway in VF-001 Phase IIb

Recruitment in the VF-001 Phase IIb trial began in December 2016. Recruitment criteria have been designed to enrich the trial for patients with VLU of moderate severity who are most likely to benefit from VF-001 therapy, by excluding subjects expected to be non-healers or rapid healers. The 168-patient double-blinded trial will compare two doses of VF-001 to placebo, in combination with standard compression bandaging. This will allow Factor to identify the most effective dose of VF-001 and to generate the data required to refile for CE mark approval in Europe from a relatively modest 168-patient trial. The company plans to update the market on recruitment in the Phase IIb trial at the end of this quarter.

Working to expand the product pipeline

While the company's primary focus is on successfully executing the Phase IIb trial, a number of projects are underway to expand the product pipeline. The nearest-term opportunity is the development of VF-001 as a treatment for diabetic foot ulcers – an IND application is being prepared to support a Phase II trial in this indication. Other projects underway include the development of a high viscosity (gel) formulation of VF-001; preclinical studies of VF-001 and other vitronectin-targeted growth factors in ocular wound healing; and a collaboration with Monash University targeting orphan skin diseases.

Valuation: Increased to A$108m or A$0.15 per share

Our valuation increases to A$108m (vs A$102m) due to the roll-forward of the risk-adjusted DCF model to end of H117 (31 December 2016). Our valuation is equal to A$0.15 per share (vs A$0.14 per share). The cash balance at 31 December was A$12.7m, which should be sufficient to fund operations beyond the reporting date for the key Phase IIb trial.

23 February 2017

Price	**A$0.07**
Market cap	**A$50m**
	US$0.76/A$
Net cash (A$m) at 31 December 2016	12.7
Shares in issue	730.0m
Free float	92%
Code	FTT
Primary exchange	ASX
Secondary exchange	N/A

Share price performance

%	1m	3m	12m
Abs	11.3	(11.5)	83.5
Rel (local)	8.6	(17.1)	58.6
52-week high/low		A$0.1	A$0.0

Business description

Factor Therapeutics is an Australian biotechnology company that specialises in the development and manufacture of biologics for advanced wound care applications. Its strategy is to use targeted growth factors to renew the wound environment and promote healing.

Next events

Update on recruitment in VLU Phase IIb	End Q117
Update on DFU IND preparations	March 2017
Top line results VLU Phase IIb	Q417

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Company description: Healing chronic wounds

Factor Therapeutics' lead product, VF-001, is an advanced biologic product for wound care that harnesses a targeted growth factor approach to accelerate healing. It is a synthetic protein combining fragments of the extracellular matrix protein vitronectin with the insulin-like growth factor 1. It was shown to be safe and well tolerated in an open-label Phase II trial in 53 patients with non-healing venous leg ulcers; in that study, one-third of ulcers healed after 12 weeks of treatment with VF-001 combined with standard compression bandaging. The company had previously applied for CE mark approval for VF-001 from the European Medicines Agency (EMA), but withdrew the application in 2015 when additional preclinical and safety data was requested.

Targeted Phase IIb clinical trial underway

Factor has achieved a significant milestone with the initiation of a 168-patient randomised, placebo-controlled Phase IIb trial of VF-001 in patients with venous leg ulcers (NCT02973893). The first subject was enrolled in December, with enrolment planned to be expanded to a total of 26 sites across the US; according to clinicaltrials.gov there were 14 sites actively recruiting patients as of 15 February. Management suggests the number of active sites is now closer to 20.

The study will test two doses of VF-001 vs placebo (randomised 1:1:1) in conjunction with standard care. The lower-dose group will receive the same 14µg dose that was used in the previous open-label Phase II trial, while the dose in the high-dose group will be 10-fold higher at 140µg. Subjects will receive weekly applications of VF-001 (or placebo) for 12 weeks in addition to standard moisture-retentive dressings and compression bandaging, and will be followed for a further 12 weeks after the completion of the treatment period.

The trial is designed to recruit patients with VLU of moderate severity, by excluding patients with less severe ulcers that are likely to heal with standard care alone as well as those with the most severe ulcers that are unlikely to fully heal within the 12-week study window despite the treatment with VF-001. The key selection criteria will be wound size and duration, which were identified as key predictors of ulcer healing by Margolis et al. (2000).[1] Subjects enrolled in the trial will have either medium-size (2.5-5cm^2) ulcers that have been present for at least six months, or large (5-15cm^2) but recent ulcers that have been present for less than six months. The selected patients will be similar to the Margolis 1 (intermediate) patients, who showed the greatest improvement in healing in the open-label VF-001 Phase II, when compared to a historical comparison group.

Subjects will also be excluded if they have other features that are likely to interfere with healing, such as diabetes, infection of the ulcer, or arterial insufficiency.

Subjects must have undergone treatment with compression therapy and moisture-retentive dressings for at least one month before the screening visit. Following screening, subjects will undergo standardised compression therapy for two weeks before the first study treatment. Patients with rapidly healing ulcers (>30% reduction in ulcer area during the run-in period) will be excluded from the trial.

The primary endpoint is the percentage reduction in ulcer area at the end of treatment. This will allow the study to identify the time when there is the greatest difference between VF-001 and placebo, which could influence the design of Phase III studies.

Secondary endpoints include the proportion of patients with complete ulcer closure after 12 weeks of treatment, which is the FDA's preferred efficacy endpoint (complete ulcer closure would be the

1 Margolis et al. Which Venous Leg Ulcers Will Heal with Limb Compression Bandages? *Am J Med*. 2000;109:15–19.

primary endpoint for subsequent US Phase III studies). Time to ulcer closure, pain reduction and improvement in quality of life are additional secondary endpoints.

Factor plans to issue an update on recruitment in late Q117, and is targeting a top-line efficacy data read-out in Q417.

Potential for CE mark filing in 2018 on Phase IIb data

The US-based Phase IIb trial has been designed to comply with the data-gathering requirements outlined by the EMA as part of the company's withdrawal of its previous application for CE mark approval in 2015. The data from the current trial could be included in a CE mark resubmission to the EMA in 2018.

Positive results in the Phase II trial would also provide evidence of efficacy to support partnering discussions and negotiations for pricing in the EU.

IND submission in diabetic foot ulcers in preparation

Factor is preparing an IND submission for a Phase II trial of VF-001 in diabetic foot ulcers (DFU). The company expects to provide an update on the preparations for the IND submission around the end of March 2017. Clinical development in DFU is a logical next step for VF-001, as most other advanced wound-care products that are approved for treating VLU are also approved for DFU, including Apligraf, Oasis Wound Matrix and ReGenerCell.

High viscosity formulation being developed

Factor is developing a high-viscosity gel formulation of the active ingredient in VF-001, which it has termed VF-002. It is anticipated that a gel formulation may increase efficacy by remaining in contact with the wound for longer. A high-viscosity formulation may also provide additional IP protection for VF-002.

Additional opportunities in orphan diseases and ocular wounds

Factor has entered a collaboration and intellectual property option agreement with a team of dermatology researchers at Monash University, led by Associate Professor Ian Smyth. The team at Monash has identified a library of anti-inflammatory small molecules that target key inflammation pathways that play a role in wound healing. Several of these molecules are candidate therapies for orphan diseases such as Harlequin ichthyosis. Harlequin ichthyosis is a severe genetic disorder that mainly affects the skin. Infants with this condition are born with very hard, thick skin covering most of their bodies.

In September 2016 Dr Robert Ryan was appointed to the board of Factor. Dr Ryan was formerly CEO of Scioderm, and has extensive experience in developing therapies for orphan skin diseases.

The company has started preclinical work for its ocular wound-care programme to assess the potential of VF-001 and other vitronectin-targeted growth factors in ocular wound healing. It expects to provide an update on the progress in this project in mid-2017.

Valuation

We have updated the cash balance to end H117 (31 December 2016) and have rolled forward our risk-adjusted discounted cash flow model for the half-year, which increases our valuation to A$108m (vs A$102m); all other valuation assumptions are unchanged. Our valuation is equal to A$0.15 per share (vs A$0.14 per share), both on an undiluted basis and after diluting for the 13.2m options on issue (exercise price 3.5-11c; all of the options would be in the money if the stock was trading in line with our valuation).

Exhibit 1 shows our (unchanged) market assumptions for VF-001 for VLU and DFU indications and the contribution of product royalties and milestone payments to the rNPV. For a detailed description of valuation assumptions see our initiation report dated 28 October 2016.

Exhibit 1: Factor Therapeutics sum-of-the-parts DCF

	Likelihood (%)	rNPV (A$m)	rNPV/ share	Assumptions
1. US VLU	30%	15.2	A$0.021	Peak sales* of US$130m in 2027 assuming 856,000 VLU cases per year in the US, 50% of ulcers are suitable for VF-001 therapy, 20% uptake among target patients; pricing of US$1,000 per patient; launch 2022; 15% royalty on net sales.
2. Europe VLU	50%	30.4	A$0.042	Peak sales of US$110m in 2025 assuming 1,315,000 VLU cases per year in Europe, 50% of ulcers are suitable for VF-001 therapy, 12% uptake among target patients; pricing of US$1,000 per patient; launch mid 2019; 15% royalty on net sales.
3. Japan and Australia VLU	40%	8.5	A$0.012	Peak sales of US$55m in 2027 assuming 597,000 VLU cases per year, 50% of ulcers are moderately severe and suitable for VF-001 therapy, 12% uptake among target patients; pricing of US$1,000 per patient; launch 2022; 15% royalty on net sales.
4. US DFU	20%	5.7	A$0.008	Peak sales of US$105m in 2029 assuming 528,000 DFU cases per year (2.4% incidence in 22 million diabetics), 12% uptake; pricing of US$1,000 per patient; launch 2024; 15% royalty on net sales.
5. Rest of world DFU	20%	5.1	A$0.007	Peak sales of US$95m in 2029 assuming 916,000 DFU cases per year (2.4% incidence in 38.2 million diabetics in eurozone, UK, Japan, Australia), 6% uptake; pricing of US$1,000 per patient; launch 2024; 15% royalty on net sales.
6. VF-001 milestones	30-70%	34.6	A$0.047	Assumes potential licensing US$30m upfronts (risked to 70%) and US$90m milestones (risked to 50-30%). Total US$120m (US$55m after risk adjustment).
7. SG&A & R&D expenses to 2020		(4.0)	(A$0.005)	
Portfolio total		95.6	A$0.131	
Cash at December 2016		12.7	A$0.017	
Enterprise total		108.2	A$0.148	

Source: Edison Investment Research. Note: *We assume that the addressable markets grow at 4% per year.

Sensitivities

The key risk for Factor is a failure to demonstrate efficacy in the Phase IIb VLU trial. A number of potential competitor products have failed to meet the primary efficacy endpoint in pivotal VLU trials. Even if the trial results are positive, there is a risk the company may not succeed in obtaining regulatory approvals, including from the US FDA and the European Medicines Authority. The FDA classifies VF-001 as a biological drug so it is likely to need to demonstrate efficacy in one or more pivotal Phase III trials to gain regulatory approval.

Factor has indicated that it intends to rigorously screen potential subjects for the Phase II trial to eliminate subjects whose VLU is on a healing trajectory after at least six weeks of standard compression therapy (at least four weeks of therapy before enrolment followed by a two-week run-in period). If these stringent criteria eliminate a high proportion of screened candidates, then recruitment could take longer than anticipated.

If the Phase IIb trial takes longer than anticipated or the company is not able to attract a suitable commercial partner then it may need to raise additional funds in the future. There is no guarantee that funds will be available at favourable terms.

Financials

We leave our financial forecasts unchanged following the release of H117 results (encompassing the six months ending 31 December 2016). Net loss in H117 was A$1.4m, comprising operating expenses of A$3.6m and other income of A$2.3m (including a A$2.2m R&D rebate). We forecast total operating expenses for FY17 of A$8.4m, which allows for expenditure to increase to A$4.8m in H217 as the recruitment commences in all 24 sites in the Phase II trial. The company has submitted amended claims for R&D rebates covering FY12-14, which are undergoing assessment by the Australian Tax Office. In our forecasts we assume that additional rebates totalling A$0.4m will be recognised in H217, taking the total recognised in FY17 to A$2.6m.

Cash at 31 December was A$12.7m, which should be sufficient to fund operations beyond the reporting date for the key Phase IIb trial.

Exhibit 2: Financial summary

A$000s	2014	2015	2016	2017e	2018e
Year end 30 June	AASB	AASB	AASB	AASB	AASB
PROFIT & LOSS					
Sales, royalties, milestones	0	0	0	0	0
Other (includes R&D tax rebate)	518	355	435	2,640	1,840
Revenue	518	355	435	2,640	1,840
R&D expenses	(1,018)	(2,529)	(2,005)	(6,600)	(4,600)
SG&A expenses	(6,381)	(4,946)	(2,545)	(1,827)	(1,923)
Other	0	0	0	0	0
EBITDA	(6,881)	(7,120)	(4,116)	(5,787)	(4,683)
Operating Profit (before GW and except.)	(6,964)	(7,201)	(4,159)	(5,804)	(4,705)
Intangible Amortisation	(68)	(68)	(68)	(56)	(50)
Exceptionals	0	(4,092)	(7,486)	0	0
Operating Profit	(7,032)	(11,361)	(11,712)	(5,860)	(4,755)
Net Interest	217	155	101	431	204
Profit Before Tax (norm)	(6,816)	(7,114)	(4,125)	(5,429)	(4,551)
Profit Before Tax (reported)	(6,816)	(11,206)	(11,611)	(5,429)	(4,551)
Tax benefit	(14)	(13)	(1)	0	0
Profit After Tax (norm)	(6,830)	(7,127)	(4,126)	(5,429)	(4,551)
Profit After Tax (reported)	(6,830)	(11,219)	(11,612)	(5,429)	(4,551)
Average Number of Shares Outstanding (m)	241.8	278.3	381.4	724.3	724.3
EPS - normalised (c)	(2.82)	(4.03)	(3.04)	(0.75)	(0.63)
EPS – diluted (c)	(2.82)	(4.03)	(3.04)	(0.75)	(0.63)
Dividend per share (A$)	0.0	0.0	0.0	0.0	0.0
BALANCE SHEET					
Fixed Assets	9,314	8,392	1,326	1,293	1,261
Intangible Assets	342	342	557	502	451
Tangible Assets	8,970	8,048	769	791	809
Investments	2	2	0	0	0
Current Assets	9,519	6,814	15,121	9,725	5,206
Stocks	1,530	649	34	0	0
Debtors	184	120	201	2,407	1,607
Cash	7,077	5,579	14,376	6,808	3,090
Other	728	466	510	510	510
Current Liabilities	(1,777)	(1,806)	(881)	(881)	(881)
Creditors	(1,247)	(1,630)	(710)	(710)	(710)
Short term borrowings	0	0	0	0	0
Other	(530)	(175)	(172)	(172)	(172)
Long Term Liabilities	(196)	(75)	(45)	(45)	(45)
Long term borrowings	0	0	0	0	0
Other long term liabilities	(196)	(75)	(45)	(45)	(45)
Net Assets	16,860	13,325	15,520	10,091	5,541
CASH FLOW					
Operating Cash Flow	(7,669)	(9,650)	(5,263)	(7,958)	(3,883)
Net Interest	173	214	115	431	204
Tax	422	392	413	0	0
Capex	(17)	(5)	(221)	(40)	(40)
Acquisitions/disposals	0	0	0	0	0
Equity Financing	10,120	8,258	14,751	0	0
Dividends	0	0	0	0	0
Other	(804)	(671)	(982)	0	0
Net Cash Flow	2,224	(1,463)	8,813	(7,567)	(3,719)
Opening net debt/(cash)	(4,862)	(7,077)	(5,579)	(14,376)	(6,808)
HP finance leases initiated	0	0	0	0	0
Other	(9)	(36)	(16)	0	0
Closing net debt/(cash)	(7,077)	(5,579)	(14,376)	(6,808)	(3,090)

Source: Company data, Edison Investment Research

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