EDISON

Abliva

Company update

IND approved by the FDA, preparing for Ph II/III

Pharma & biotech

With the investigational new drug (IND) application approved by the FDA, Abliva is getting ready for the pivotal Phase II/III trial for its lead drug candidate KL1333, NAD+ modulator expected to increase cellular energy production in primary mitochondrial disorder (PMD) patients. Phase Ia/b data with first findings from treating patients were published in 2021. Abliva is now focused on finalising the regulatory approvals in other countries. Another major focus for the management is to establish funding for the Phase II/III study, which should start sometime in 2022. The recently announced planned convertible loan should provide bridge funding until then. Our valuation is little changed at SEK1.25bn or SEK3.11/share.

Year end	Revenue (SEKm)	PBT* (SEKm)	EPS* (SEK)	DPS (SEK)	P/E (x)	Yield (%)
12/19	3.6	(74.6)	(0.43)	0.0	N/A	N/A
12/20	1.9	(57.4)	(0.23)	0.0	N/A	N/A
12/21e	0.2	(110.4)	(0.32)	0.0	N/A	N/A
12/22e	0.2	(128.6)	(0.32)	0.0	N/A	N/A

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

IND approval paves the way for the Phase II/III trial

In November 2021, the FDA approved Abliva's IND application for KL1333, which paves the way for the initiation of the pivotal Phase II/III trial, recently named FALCON. This will be the first time Abliva's trial will enrol US-based patients. The fact that the FDA agreed with the IND data package is a good indication KL1333 is ready for the pivotal trial and other regulators in Europe will likely have no issues with the proposed trial design. The next step is to finalise regulatory discussions in other countries where the trial will be recruiting patients.

Trial design details revealed

The FALCON study will enrol up to 180 PMD patients, who will be randomised to receive treatment with KL1333 or placebo twice daily for 12 months. The trial results should be known in 2024. There will be two endpoints, a fatigue endpoint and a functional endpoint. In 2021, Abliva completed a qualitative study focused on validating a patient-reported outcome for fatigue that is sensitive to the PMD patient condition (there were no such scales before) and has proposed it to the FDA. Although it took some time to complete the qualitative study after the last KL1333 data were reported in May 2021, it is key that the regulator accepts the endpoints, otherwise the whole trial could be compromised.

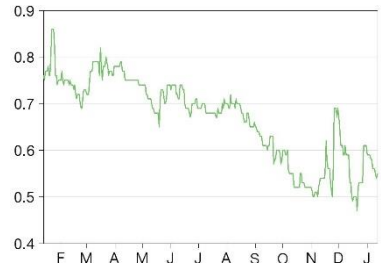
Valuation: SEK1.25bn or SEK3.11 per share

Our valuation is SEK1.25bn or SEK3.11 per share after making no major changes since we last published. Abliva has indicated it will seek new funds early this year. One of management's major focuses is initiating conversations with banks, investors and potential partners to raise the financing for the Phase II/III study. A convertible loan of SEK26m should provide bridging funding until then (an extraordinary general meeting, EGM, to consider the approval of the issue directed to the main shareholder is to be held on January 14). In our model we include $30m for the Phase II/III study. Our peak sales projections for KL1333 are $670m, but a sensitivity analysis (see below) shows it also has blockbuster potential.

13 January 2022

Price	**SEK0.55**
Market cap	**SEK222m**
Net cash (SEKm) end-Q321	65.1
Shares in issue	403m
Free float	80%
Code	ABLI
Exchange	Nasdaq Stockholm

Share price performance



%	1m	3m	12m
Abs	(7.0)	0.1	(27.0)
Rel (local)	(7.2)	(7.3)	(42.4)
52-week high/low	SEK0.9	SEK0.5	

Business description

Abliva is a Swedish biotech company with deep expertise in mitochondrial medicine. Its focus area is primary mitochondrial diseases with lead assets KL1333, an NAD+ modulator (Phase II/III ready), and NV354, a succinate prodrug (preclinical). It plans to start a pivotal Phase II/III trial with KL1333 in selected primary mitochondrial disorders this year.

Next events

Updates on the preparations for the Phase II/III for KL1333	H122
Updates on the next development steps for Phase I ready NV354	2022
EGM to approve the convertible loan	14 January 2022
Updates on funding round for the Phase II/II trial with KL1333	H122

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Edison profile page

Flagship Phase II/III FALCON trial with KL1333 to start

The IND application for KL1333 approved

The FALCON study will be the first time an Abliva trial has enrolled US-based patients. Given the unified healthcare provision and a relatively large pool of mitochondrial disease patients, the United States is a strategically important market for any biotech employing an orphan drug development strategy. The fact that the FDA agreed with the IND data package is a good indication KL1333 is ready for the pivotal trial and other regulators in Europe will likely have no issues with the proposed trial design (the key regulators are the European Medicines Agency and from the UK's Medicines and Healthcare Products Regulatory Agency).

Exhibit 1: Status of the core assets

Product	Stage	Indication	Status and upcoming events
KL1333 NAD+ modulator oral; chronic treatment	Phase II/III-ready	MIDD-MELAS, KSS-CPEO and MERRF	In-licensed from Yungjin Pharm (South Korea) in May 2017. Phase I development finished. The IND was approved by the FDA in November 2021. The trial is expected to start in 2022. Orphan drug designation.
NV354 succinate prodrug iv and oral alternative energy source	Phase I ready	Complex I disorders	Preclinical development is completed and the drug candidate could enter clinical development in 2022.

Source: Edison Investment Research, Abliva

Trial design

The clinical trial design has not been published yet, but Abliva has provided some details. The company strategically chose to target three specific mitochondrial conditions: MIDD-MELAS (encephalomyopathy, lactic acidosis and stroke-like episodes), KSS-CPEO (progressive paralysis of certain eye muscles, pigmentary retinopathy, cardiomyopathy and arrhythmia) and MERRF (myoclonic epilepsy, ataxia, weakness and dementia) syndromes. Although the symptoms of these syndromes vary significantly depending on the organ system affected, all patients will have chronic fatigue and muscle weakness (a detailed discussion about the biology of mitochondria, energy production and PMDs is in our last published report).

This focus will allow the investigators to evaluate the primary endpoints in a more homogenous patient population, which should improve the statistical analysis. However, we note that KL1333 theoretically could be beneficial in other PMD conditions as well; PMD is a group of diverse conditions and all are rare or ultrarare. So, if the efficacy is proven in MIDD-MELAS, KSS-CPEO and MERRF, label expansion with bridging trials in other conditions is possible.

The FALCON study will be a randomised, double-blind and placebo-controlled trial in up to 180 patients, who will be randomised to receive peroral treatment with KL1333 twice daily or placebo (60% will receive KL1333 and 40% will receive placebo) for 12 months. The study timeline envisages:

- patient enrolment starts in 2022;
- interim futility analysis is planned in 2023; and
- data readout in 2024.

There will be two endpoints, a fatigue endpoint and a functional endpoint (30-second sit to stand test). Because PMDs are very rare conditions with no effective treatment, there are no standard measures to evaluate the efficacy of any potential new drug. Of the many different symptoms PMD patients experience, Abliva has identified fatigue as the most common and one that could be reliably quantifiable. The FDA also agreed this symptom can be used for the primary endpoint. In 2021, Abliva conducted a qualitative study focused on validating a patient reported outcome for fatigue that is sensitive to the PMD patient condition. There are other fatigue scales recognised by

regulators, however, Abliva did not want to use these off-the-shelf solutions and has developed its own, with hopes that this will increase the specificity. We note that Abliva has a long history of working with PMD patient associations (eg World Mitochondrial Disease Week 2021 event), which, in our view, is key to understanding the day-to-day challenges PMD patients face. This, we believe, will ensure the informed selection of an appropriate endpoint for the Phase II/III trial.

The next step now the IND is approved in the United States is to finalise regulatory discussions in other countries where the trial will be recruiting patients.

Financials and valuation

From a funding perspective, Abliva has indicated, in conjunction with the December 2021 resolution proposing the issue of a convertible loan through a directed issue of convertible bonds, it will aim to seek new funds early in 2022. In its Q321 report, management also stated a major focus is initiating conversations with banks, investors and potential partners to raise the financing for the Phase II/III study. In our model we include $30m for the Phase II/III study. There are approximately 40k patients in the target group for KL1333 in Europe and the United States. Assuming an orphan drug price tag ($110k in the United States, 50% discount in Europe) and a 20% market penetration we calculate KL1333's peak sales of $670m. Given the lack of benchmark drugs, the market penetration is an uncertain estimate. Presuming KL1333 shows a good clinical effectiveness, the penetration could be much higher. Exhibit 3 provides a sensitivity analysis of KL1333 peak sales. More detailed discussion about our valuation assumptions can be found in our last published report.

According to Abliva's latest quarterly report, in 9M21 the operating loss was SEK86.6m, higher year-on-year (SEK46.9m in 9M20) due to increased R&D spending. We forecast a FY21 operating loss of SEK113.0m, which should increase further in the coming years as clinical trials are initiated for both assets (our FY22 and FY23 operating loss estimates are SEK128.6m and SEK136.3m, respectively).

At end-Q321, cash was SEK65.1m. In Q421 Abliva announced a planned issue of convertible bonds amounting to SEK26m directed to the company's largest shareholder Hadean Ventures. The convertibles carry an annual interest rate of 10% of their nominal value. The term is 12 months with a maturity date of 20 December 2022.

The announcement also states, 'the conversion price will either be (i) same price as other investors in a potential capital raise conducted before 22 May 2022, or (ii) 10-day VWAP prior to conversion request date'. This indicates the loan serves as bridge financing to the potential share issue sometime in H122. At full conversion calculated as of the date of the announcement, the dilution would amount to approximately 10.7% (using 10-day VWAP). The issue is still subject to investor approval (an EGM is due on 14 January 2022).

Our valuation is SEK1.25bn or SEK3.11 per share, slightly higher than our previous SEK1.21bn or SEK3.11 per share as rolling the model forward has offset the lower cash. We do not yet include the convertible loan in our valuation model, which is underpinned by the two lead assets KL1333 and NV354.

Exhibit 2: Abliva sum-of-the parts valuation

Product	Launch	Peak sales ($m)	NPV (SEKm)	NPV/share (SEK)	Probability (%)	rNPV (SEKm)	rNPV/share (SEK)
Core assets							
KL1333	2025	670	4,631.2	11.49	25%	1,106.3	2.75
NV354	2028	470	2,913.1	7.23	5%	80.5	0.20
Net cash, last reported (Q321)			65.1	0.16	100%	65.1	0.16
Valuation			**7,609.3**	**18.9**		**1,251.9**	**3.11**

Source: Edison Investment Research. Note: WACC = 12.5% for product valuations.

Exhibit 3: Peak sales sensitivity to market penetration and pricing

KL1333		Market penetration				
		10%	20%	40%	60%	80%
Pricing	60,000	180	360	730	1,090	1,460
	110,000	330	**670**	1,340	2,010	2,670
	160,000	490	970	1,950	2,920	3,890

NV354		Market penetration				
		10%	20%	30%	60%	80%
Pricing	80,000	100	190	290	580	780
	130,000	160	320	**470**	950	1,260
	180,000	220	440	660	1,310	1,750

Source: Edison Investment Research

Exhibit 4: Financial summary

SEK'000s	2019	2020	2021e	2022e
Year end 31 December	IFRS	IFRS	IFRS	IFRS
PROFIT & LOSS				
Revenue	3,634	1,864	200	200
Cost of Sales	0	0	0	0
Gross Profit	3,634	1,864	200	200
EBITDA	(72,317)	(54,955)	(107,865)	(126,102)
Operating Profit (before amort. and except.)	(74,696)	(57,513)	(110,400)	(128,640)
Intangible Amortisation	0	0	0	0
Exceptionals	(2,379)	(2,558)	(2,558)	0
Other	0	0	0	0
Operating Profit	(77,075)	(60,071)	(112,958)	(128,640)
Net Interest	75	77	0	0
Profit Before Tax (norm)	(74,621)	(57,436)	(110,400)	(128,640)
Profit Before Tax (reported)	(77,000)	(59,994)	(112,958)	(128,640)
Tax	0	0	0	0
Minority Interests	(6)	(5)	(5)	(5)
Profit After Tax (norm)	(74,621)	(57,436)	(110,400)	(128,640)
Profit After Tax (reported)	(76,994)	(59,989)	(112,953)	(128,635)
Average Number of Shares Outstanding (m)	171.6	249.7	349.6	403.0
EPS - normalised (SEK)	(0.43)	(0.23)	(0.32)	(0.32)
EPS - normalised fully diluted (SEK)	(0.43)	(0.23)	(0.32)	(0.32)
EPS - reported (SEK)	(0.45)	(0.24)	(0.32)	(0.32)
Dividend per share (SEK)	0.0	0.0	0.0	0.0
Gross Margin (%)	100.0	100.0	100.0	100.0
EBITDA Margin (%)	N/A	N/A	N/A	N/A
Operating Margin (before GW and except.) (%)	N/A	N/A	N/A	N/A
BALANCE SHEET				
Fixed Assets	88,573	87,506	87,579	87,255
Intangible Assets	74,686	74,021	74,094	73,770
Tangible Assets	99	41	41	41
Investments	13,788	13,444	13,444	13,444
Current Assets	59,919	63,157	30,126	3,514
Stocks	0	0	0	0
Debtors	0	0	0	0
Cash	58,319	61,643	28,612	2,000
Other	1,600	1,514	1,514	1,514
Current Liabilities	(20,337)	(10,209)	(10,209)	(10,209)
Creditors	(20,337)	(10,209)	(10,209)	(10,209)
Short term borrowings	0	0	0	0
Long Term Liabilities	(361)	(92)	(92)	(101,796)
Long term borrowings	0	0	0	(101,704)
Other long term liabilities	(361)	(92)	(92)	(92)
Net Assets to shareholders and minority interests	127,794	140,362	107,404	(21,236)
CASH FLOW				
Operating Cash Flow	(72,367)	(67,528)	(110,423)	(126,102)
Net Interest	(46)	(30)	0	0
Tax	0	0	0	0
Capex	(69)	0	0	0
Acquisitions/disposals*	0	0	0	0
Financing	107,780	72,564	80,000	0
Other	(2,930)	(1,682)	(2,608)	(2,214)
Dividends	0	0	0	0
Net Cash Flow	32,368	3,324	(33,031)	(128,316)
Opening net debt/(cash)	(25,951)	(58,319)	(61,643)	(28,612)
HP finance leases initiated	0	0	0	0
Other	(0)	0	0	0
Closing net debt/(cash)	(58,319)	(61,643)	(28,612)	99,704

Source: Abliva accounts, Edison Investment Research

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