

# Kiadis Pharma

## One-year follow-up data presented at ASH

Kiadis Pharma has presented one-year follow-up data from a Phase II study in leukaemia patients (n=23) at the 58<sup>th</sup> meeting of the American Society of Hematology (ASH) in San Diego, US. Patients were administered ATIR101 after a haematopoietic stem cell transplant (HSCT) from a partially matching donor. The primary endpoint of transplant-related mortality (TRM) and secondary endpoint overall survival (OS) for patients receiving HSCT+ATIR101 were significantly higher than patients receiving HSCT alone from an observational control group. We are encouraged to see the low incidence of relapse in the ATIR101 arm in this high-risk patient population. Our valuation is €383.2m or €27.4 per share.

| Year end | Revenue (€m) | PBT* (€m) | EPS* (€) | DPS (€) | P/E (x) | Yield (%) |
|----------|--------------|-----------|----------|---------|---------|-----------|
| 12/14    | 0.00         | (7.21)    | (0.07)   | 0.0     | N/A     | N/A       |
| 12/15    | 0.00         | (17.35)   | (0.14)   | 0.0     | N/A     | N/A       |
| 12/16e   | 0.00         | (9.99)    | (0.07)   | 0.0     | N/A     | N/A       |
| 12/17e   | 0.00         | (13.48)   | (0.10)   | 0.0     | N/A     | N/A       |

Note: \*PBT and EPS are normalised, excluding intangible amortisation, exceptional items and share-based payments.

## ATIR101: Increasingly positive data at one year

In this open-label single dose Phase II study, ATIR101 administered after HSCT showed a significant reduction in the primary endpoint of TRM at 32% vs 70% for HSCT only (p=0.007) of a historical comparator group. Overall survival was 61% for HSCT+ATIR101 vs 20% for HSCT only (p=0.0023). There were no cases of grade III-IV acute Graft vs Host Disease (GVHD), only three cases of grade II acute GVHD, and one chronic GVHD. Thus, event-free survival rate (GRFS) was 57% after one year, which is higher than 20% for the historic group, even for matching unrelated donors (41%) and post-transplant cyclophosphamide (PTCy) of 33% as reported by Solh et al. (2016).

## Up next: Start Phase III trial and EU filing

Kiadis plans to initiate a Phase III trial in patients with acute leukaemia soon after regulatory approval of the protocol (we assume Q117). Patients will be randomised to receive a single dose of ATIR101 or PTCy after haploidentical HSCT. Furthermore, on the back of the Phase II data, the company plans to submit a Marketing Authorization Application for conditional approval to the European Medicines Agency (EMA) in Q117. ATIR201 will start clinical testing in thalassemia in H216.

## Valuation: Upgraded to €383.2m from €327.3m

We are slightly increasing the probability of success for ATIR101 in the EU from 70% to 75% to reflect our positive stance on Kiadis receiving EMA approval based on positive Phase II data and the precedent of MolMed, which received conditional approval on lower one-year survival data of 49%. We are also rolling the valuation forward in time. This results in an increased rNPV valuation of €383.2m vs previous €327.3m.

Clinical data update

Pharma & biotech

8 December 2016

**Price** €8.98

**Market cap** €125m

US\$1.11/€

Net cash (€m) at June 2016 (excludes Hospira debt) 16.2

Shares outstanding 13.97m

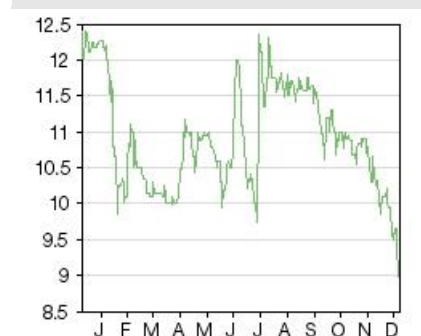
Free float 31.1%

Code KDS

Primary exchange Euronext Amsterdam

Secondary exchange Euronext Brussels

### Share price performance



% 1m 3m 12m

Abs (15.7) (19.9) (26.3)

Rel (local) (18.3) (19.7) (28.5)

52-week high/low €12.4 €9.0

### Business description

Kiadis Pharma is a Dutch biotech company focused on cell-based immunotherapies to overcome complications associated with stem cell transplants in blood diseases. Lead product ATIR101 for leukaemia is undergoing a Phase II trial and will file for EU approval in Q117. ATIR201 is in preclinical stage and has potential for thalassemia. A Phase I/II study will start in H216.

### Next events

ATIR101 Phase III start H216

ATIR201 Phase I/II start H216

ATIR101 EU filing Q117

ATIR101 two-year data H217

### Analysts

Juan Pedro Serrate +44 (0)20 3681 2534

Lala Gregorek +44 (0)20 3681 2527

[healthcare@edisongroup.com](mailto:healthcare@edisongroup.com)

[Edison profile page](#)

**Kiadis Pharma is a research client of Edison Investment Research Limited**

## One-year Phase II data confirms efficacy of ATIR101

Kiadis Pharma has presented one-year data of its ongoing Phase II trial in patients with acute leukaemia subject to HSCT at the 58<sup>th</sup> meeting of the ASH. This is a multicentre, open-label Phase II trial ([NCT01794299](#), CR-AIR-007) evaluating the safety and efficacy of a T-cell depleted haploidentical transplant followed by ATIR101 infusion at a median of 28 days post-transplant. The primary endpoint is TRM; relevant secondary endpoints are OS and progression-free survival. The trial included 23 patients (16 with acute myeloid leukaemia and seven with acute lymphoblastic leukaemia) who were in first or second remission. Most patients (57%) had a high or very high risk of leukaemia; the rest had an intermediate risk. Patients did not receive post-transplant GVHD prophylaxis.

There was no TRM after 100 days of treatment. There were seven deaths in the ATIR101 arm as a result of TRM at one year post-HSCT, resulting in a TRM rate of 32%. All seven patients died from infection. In addition, two patients died from disease relapse, resulting in an overall survival of 61%. There were no cases of grade III-IV acute GVHD and only three of grade II acute GVHD. One patient had chronic GVHD.

**Exhibit 1: Phase II clinical data at one-year follow up**

| Endpoint                | HSCT+ ATIR101 | HSCT only |
|-------------------------|---------------|-----------|
| TRM                     | 32%           | 70%       |
| OS                      | 61%           | 20%       |
| GFRS                    | 57%           | 20%       |
| Grade III/IV acute GVHD | 0%            | N.D.      |
| Grade II acute GVHD     | 13%           | N.D.      |
| Chronic GVHD            | 4.3%          | N.D.      |

Source: Edison Investment Research, Kiadis Pharma. Note: N.D. = not disclosed.

These data compare favourably with a historic control group obtained from the same centres and with the same inclusion/exclusion criteria that had undergone a transplant from haploidentical family members ([NCT02188290](#)/CR-AIR-006). When compared to the control group (n=35), TRM was significantly lower in patients given ATIR101 after a T-cell depleted haplo HSCT with a one-year TRM of 32% versus 70% for HSCT only (p=0.007). Overall survival was significantly higher in the HSCT+ATIR101 group (61%), compared to HSCT alone (p=0.0023).

Event-free survival, defined as GVHD-free, relapse-free survival (GFRS<sup>1</sup>), was 57% after one year in the HSCT+ATIR101 group, which is higher than 20% for the HSCT-only group. Interestingly, this result is also higher than matching unrelated donors (41%) in the same group. According to [Sol\(h et al. \(2016\)\)](#), patients receiving cyclophosphamide after haploidentical HSCT had a GFRS of 33%.

## EU registration and Phase III plans on track

We expect the full Phase III trial to start in Q117. The protocol has been submitted to regulatory authorities in the EU and US and is currently under review for approval. Patients will receive either ATIR101 or the Baltimore approach (PTCy) after haploidentical HSCT. The primary endpoint of the Phase III trial will be GFRS. Moreover, Kiadis anticipates an EU regulatory filing in Q117 on the back of the Phase II results. Additionally, ATIR101 is undergoing a Phase II study in which a second dose of the product is administered to patients before the 100-day period post-transplantation to support faster immune-reconstitution. The full enrolment and safety readout will be completed in H217.

<sup>1</sup> It is a composite endpoint that measures survival free of morbidity defined as grade III-IV aGVHD, cGVHD requiring systemic treatment, cancer relapse, or death.

The second product, ATIR201 for thalassemia, is currently in a preclinical stage. A Phase I/II programme is slated to start H217. The development pipeline is summarised in Exhibit 2.

**Exhibit 2: Kiadis Pharma product portfolio**

| Product | Indication  | Status      | Potential launch | Comments  |
|---------|-------------|-------------|------------------|---|
| ATIR101 | Leukaemia   | Phase II    | 2018             | Presented positive data at six months and one year. Regulatory filing in EU in Q117. Phase III start in Q117. |
| ATIR201 | Thalassemia | Preclinical | 2022             | Preclinical stage. Start Phase I/II trial in H216.  |

Source: Edison Investment Research, Kiadis Pharma

## Valuation: Increasing to €383.2m or €27.4/share

We are upgrading our rNPV valuation to €383.2m or €27.4 per share vs our previous €327.3m. We are slightly increasing the probability of receiving EMA approval for ATIR101 from 70% to 75% based on positive Phase II data and following the precedent of MolMed, which received conditional approval on lower one-year survival data of 49%. We are also rolling the valuation forward by half a year. Our underlying financial forecasts remain unchanged.

We are not including ATIR201 in the valuation as it is in the preclinical stage. We will revisit our rNPV model once clinical data are released.

**Exhibit 3: Kiadis Pharma rNPV valuation**

| Product                 | Indication | Launch | Peak sales (US\$m) | Probability | rNPV (€m)    | rNPV/share (€) |
|-------------------------|------------|--------|--------------------|-------------|--------------|----------------|
| ATIR101 EU              | Leukaemia  | 2018   | 227.8              | 75%         | 351.9        | 25.2           |
| ATIR101 US              | Leukaemia  | 2020   | 273.3              | 50%         | 237.9        | 17.0           |
| Expenses                |            |        |                    |             | 214.4        | 15.4           |
| Net cash June 2016*     |            |        |                    |             | 7.7          | 0.6            |
| <b>Number of shares</b> |            |        |                    |             |              | <b>13.97m</b>  |
| <b>Valuation</b>        |            |        |                    |             | <b>383.2</b> | <b>27.4</b>    |

Source: Edison Investment Research. Note: \*Includes Hospira debt.

**Exhibit 4: Financial summary**

|  | €'000s | 2013     | 2014    | 2015     | 2016e    | 2017e    | 2018e    |
|--|--------|----------|---------|----------|----------|----------|----------|
| Year end 31 December                         |        | IFRS     | IFRS    | IFRS     | IFRS     | IFRS     | IFRS     |
| <b>PROFIT &amp; LOSS</b>                     |        |          |         |          |          |          |          |
| Revenue                                      |        | 0        | 0       | 0        | 0        | 0        | 3,906    |
| Cost of Sales                                |        | 0        | 0       | 0        | 0        | 0        | (1,953)  |
| Gross Profit                                 |        | 0        | 0       | 0        | 0        | 0        | 1,953    |
| R&D expenses                                 |        | (3,548)  | (4,692) | (7,715)  | (7,230)  | (10,000) | (10,000) |
| SG&A expenses                                |        | (1,444)  | (1,476) | (8,292)  | (1,476)  | (2,000)  | (4,000)  |
| EBITDA                                       |        | (4,890)  | (6,042) | (15,867) | (8,606)  | (11,900) | (12,142) |
| Operating Profit (before GW and except.)     |        | (4,992)  | (6,168) | (16,007) | (8,706)  | (12,000) | (12,242) |
| Intangible Amortisation                      |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Exceptionals/Other                           |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Operating Profit                             |        | (4,992)  | (6,168) | (16,007) | (8,706)  | (12,000) | (12,242) |
| Net Interest                                 |        | (831)    | (1,045) | (1,344)  | (1,284)  | (1,476)  | (1,473)  |
| Exceptionals                                 |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Other  |        | (1,062)  | (598)   | 894      | 0        | 0        | 0        |
| Profit Before Tax (norm)                     |        | (5,823)  | (7,213) | (17,351) | (9,990)  | (13,476) | (13,715) |
| Profit Before Tax (IFRS)                     |        | (6,885)  | (7,811) | (16,457) | (9,990)  | (13,476) | (13,715) |
| Tax  |        | 0        | (2)     | (1)      | 0        | 0        | 0        |
| Discontinued operations                      |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Profit After Tax (norm)                      |        | (6,885)  | (7,813) | (16,458) | (9,990)  | (13,476) | (13,715) |
| Profit After Tax (IFRS)                      |        | (6,885)  | (7,813) | (16,458) | (9,990)  | (13,476) | (13,715) |
| Average Number of Shares Outstanding (m)     |        | 10.90    | 10.47   | 12.06    | 13.97    | 13.97    | 13.97    |
| EPS - normalised (€)                         |        | (0.06)   | (0.07)  | (0.14)   | (0.07)   | (0.10)   | (0.10)   |
| EPS - IFRS (€)                               |        | (0.06)   | (0.07)  | (0.14)   | (0.07)   | (0.10)   | (0.10)   |
| Dividend per share (€)                       |        | 0.00     | 0.00    | 0.00     | 0.00     | 0.00     | 0.00     |
| Gross Margin (%)                             |        | N/A      | N/A     | N/A      | N/A      | N/A      | N/A      |
| EBITDA Margin (%)                            |        | N/A      | N/A     | N/A      | N/A      | N/A      | N/A      |
| Operating Margin (before GW and except.) (%) |        | N/A      | N/A     | N/A      | N/A      | N/A      | N/A      |
| <b>BALANCE SHEET</b>                         |        |          |         |          |          |          |          |
| Fixed Assets                                 |        | 13,428   | 14,100  | 13,047   | 13,047   | 13,047   | 13,047   |
| Intangible Assets                            |        | 13,148   | 13,687  | 12,714   | 12,614   | 12,514   | 12,414   |
| Tangible Assets                              |        | 280      | 413     | 333      | 433      | 533      | 633      |
| Other  |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Current Assets                               |        | 6,760    | 6,112   | 29,229   | 19,239   | 17,763   | 3,852    |
| Stocks                                       |        | 0        | 0       | 0        | 0        | 0        | 98       |
| Debtors                                      |        | 51       | 196     | 145      | 145      | 145      | 391      |
| Cash   |        | 6,482    | 5,674   | 28,666   | 18,676   | 17,200   | 2,946    |
| Other  |        | 227      | 242     | 418      | 418      | 418      | 418      |
| Current Liabilities                          |        | (1,619)  | (8,727) | (2,913)  | (1,747)  | (1,746)  | (1,745)  |
| Creditors                                    |        | (1,235)  | (1,598) | (1,747)  | (1,747)  | (1,747)  | (1,747)  |
| Short term borrowings                        |        | (384)    | (7,129) | (1,166)  | 0        | 0        | 0        |
| Deferred revenues                            |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Other short term liabilities                 |        | 0        | 0       | 0        | 0        | 1        | 2        |
| Long Term Liabilities                        |        | (13,210) | (8,820) | (13,713) | (14,879) | (26,879) | (26,684) |
| Long term borrowings                         |        | (10,021) | (5,090) | (13,713) | (14,879) | (26,879) | (26,684) |
| Deferred revenues                            |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Other long term liabilities                  |        | (3,189)  | (3,730) | 0        | 0        | 0        | 0        |
| Net Assets                                   |        | 5,359    | 2,665   | 25,650   | 15,660   | 2,185    | (11,529) |
| <b>CASH FLOW</b>                             |        |          |         |          |          |          |          |
| Operating Cash Flow                          |        | (4,369)  | (6,062) | (7,955)  | (8,606)  | (11,900) | (12,485) |
| Net Interest                                 |        | (28)     | (13)    | (141)    | (1,284)  | (1,476)  | (1,473)  |
| Tax  |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Capex  |        | (102)    | (259)   | (59)     | (100)    | (100)    | (100)    |
| Acquisitions/disposals                       |        | 0        | 0       | 1        | 2        | 3        | 4        |
| Financing                                    |        | 0        | 5,051   | 31,229   | 0        | 0        | 0        |
| Dividends                                    |        | 0        | 0       | 0        | 0        | 0        | 0        |
| Other  |        | 89       | 28      | 4        | 0        | 0        | 0        |
| Net Cash Flow                                |        | (4,410)  | (1,255) | 23,079   | (9,988)  | (13,473) | (14,055) |
| Opening net debt/(cash)                      |        | (505)    | 3,923   | 6,545    | (13,787) | (3,797)  | 9,679    |
| HP finance leases initiated                  |        | 0        | 0       | 0        | 0        | 0        | 1        |
| Exchange rate movements                      |        | 25       | (8)     | 22       | 0        | 0        | 0        |
| Other  |        | (43)     | (1,359) | (2,769)  | (2)      | (3)      | (5)      |
| Closing net debt/(cash)                      |        | 3,923    | 6,545   | (13,787) | (3,797)  | 9,679    | 23,738   |

Source: Edison Investment Research, Kiadis Pharma accounts

Edison, the investment intelligence firm, is the future of investor interaction with corporates. Our team of over 100 analysts and investment professionals work with leading companies, fund managers and investment banks worldwide to support their capital markets activity. We provide services to more than 400 retained corporate and investor clients from our offices in London, New York, Frankfurt, Sydney and Wellington. Edison is authorised and regulated by the [Financial Conduct Authority](#). Edison Investment Research (NZ) Limited (Edison NZ) is the New Zealand subsidiary of Edison. Edison NZ is registered on the New Zealand Financial Service Providers Register (FSP number 247505) and is registered to provide wholesale and/or generic financial adviser services only. Edison Investment Research Inc (Edison US) is the US subsidiary of Edison and is regulated by the Securities and Exchange Commission. Edison Investment Research Limited (Edison Aus) [46085869] is the Australian subsidiary of Edison and is not regulated by the Australian Securities and Investment Commission. Edison Germany is a branch entity of Edison Investment Research Limited [4794244]. [www.edisongroup.com](http://www.edisongroup.com)

#### DISCLAIMER

Copyright 2016 Edison Investment Research Limited. All rights reserved. This report has been commissioned by Kiadis Pharma and prepared and issued by Edison for publication globally. All information used in the publication of this report has been compiled from publicly available sources that are believed to be reliable, however we do not guarantee the accuracy or completeness of this report. Opinions contained in this report represent those of the research department of Edison at the time of publication. The securities described in the Investment Research may not be eligible for sale in all jurisdictions or to certain categories of investors. This research is issued in Australia by Edison Aus and any access to it, is intended only for "wholesale clients" within the meaning of the Australian Corporations Act. The Investment Research is distributed in the United States by Edison US to major US institutional investors only. Edison US is registered as an investment adviser with the Securities and Exchange Commission. Edison US relies upon the "publishers' exclusion" from the definition of investment adviser under Section 202(a)(11) of the Investment Advisers Act of 1940 and corresponding state securities laws. As such, Edison does not offer or provide personalised advice. We publish information about companies in which we believe our readers may be interested and this information reflects our sincere opinions. The information that we provide or that is derived from our website is not intended to be, and should not be construed in any manner whatsoever as, personalised advice. Also, our website and the information provided by us should not be construed by any subscriber or prospective subscriber as Edison's solicitation to effect, or attempt to effect, any transaction in a security. The research in this document is intended for New Zealand resident professional financial advisers or brokers (for use in their roles as financial advisers or brokers) and habitual investors who are "wholesale clients" for the purpose of the Financial Advisers Act 2008 (FAA) (as described in sections 5(c) (1)(a), (b) and (c) of the FAA). This is not a solicitation or inducement to buy, sell, subscribe, or underwrite any securities mentioned or in the topic of this document. This document is provided for information purposes only and should not be construed as an offer or solicitation for investment in any securities mentioned or in the topic of this document. A marketing communication under FCA Rules, this document has not been prepared in accordance with the legal requirements designed to promote the independence of investment research and is not subject to any prohibition on dealing ahead of the dissemination of investment research. Edison has a restrictive policy relating to personal dealing. Edison Group does not conduct any investment business and, accordingly, does not itself hold any positions in the securities mentioned in this report. However, the respective directors, officers, employees and contractors of Edison may have a position in any or related securities mentioned in this report. Edison or its affiliates may perform services or solicit business from any of the companies mentioned in this report. The value of securities mentioned in this report can fall as well as rise and are subject to large and sudden swings. In addition it may be difficult or not possible to buy, sell or obtain accurate information about the value of securities mentioned in this report. Past performance is not necessarily a guide to future performance. Forward-looking information or statements in this report contain information that is based on assumptions, forecasts of future results, estimates of amounts not yet determinable, and therefore involve known and unknown risks, uncertainties and other factors which may cause the actual results, performance or achievements of their subject matter to be materially different from current expectations. For the purpose of the FAA, the content of this report is of a general nature, is intended as a source of general information only and is not intended to constitute a recommendation or opinion in relation to acquiring or disposing (including refraining from acquiring or disposing) of securities. The distribution of this document is not a "personalised service" and, to the extent that it contains any financial advice, is intended only as a "class service" provided by Edison within the meaning of the FAA (ie without taking into account the particular financial situation or goals of any person). As such, it should not be relied upon in making an investment decision. To the maximum extent permitted by law, Edison, its affiliates and contractors, and their respective directors, officers and employees will not be liable for any loss or damage arising as a result of reliance being placed on any of the information contained in this report and do not guarantee the returns on investments in the products discussed in this publication. FTSE International Limited ("FTSE") © FTSE 2016. "FTSE®" is a trade mark of the London Stock Exchange Group companies and is used by FTSE International Limited under license. All rights in the FTSE indices and/or FTSE ratings vest in FTSE and/or its licensors. Neither FTSE nor its licensors accept any liability for any errors or omissions in the FTSE indices and/or FTSE ratings or underlying data. No further distribution of FTSE Data is permitted without FTSE's express written consent.