Kiadis Pharma (KDS.AS)

Kiadis on Track for Key Upcoming Catalysts

Kiadis Pharmaceuticals (AMS: KDS) is a clinical stage biopharmaceutical company developing products to make hematopoietic stem cell transplantation (HSCT) safer and more effective for patients with either hematological malignancies or inherited blood disorders. The Company's lead clinical candidate is ATIR101, a donor lymphocyte infusion that is given after an allogeneic HSCT (allo-HSCT) to prevent GvHD and mitigate the risk of disease relapse and infection. A Marketing Authorization Application (MAA) for the approval of ATIR101 in Europe has been submitted and accepted for review, and Kiadis is currently responding to the Day 120 List of Questions that it has received from the EMA. The Company expects a regulatory decision from the EMA in the second half of 2018, which could pave the way for a potential launch in Europe in 2019. The Company also expects to begin enrolling patients this quarter into a Phase III study evaluating a composite measurement of GvHD and relapse-free survival in patients treated with either ATIR101 or post-transplant cyclophosphamide (PT-Cy) after receiving a half-matched HSCT.

- **Recent Financing Increases Cash Runway into 2019.** Kiadis has extended its cash runway through a private placement and debt financing. In October 2017, Kiadis received €18 ($20.9) million in gross proceeds from a private placement through the sale of 2.25 million new shares at €8.00 ($9.44) per share. In addition, Kiadis obtained debt financing of up to €15 ($17.6) million from Kreos Capital in August 2017. The first tranche consists of €10 ($11.8) million, which has been drawn down, and a second tranche of €5 ($5.8 million), which would become available once the Company raised €20 ($23.5) million in additional funds. This recent raise, along with the exercise of outstanding warrants totaling €2.3 ($2.7) million, has allowed Kiadis to access the €5 ($5.8) million from the second tranche. The Company has guided that the capital raise will be enough to fund operations into 2019.

- **Kiadis Receives List of Questions from EMA on Marketing Authorization Application.** In September of this year, the Company announced an update concerning its MAA for ATIR101 in Europe, which was submitted in April 2017. Kiadis received a Day 120 List of Questions from the European Medicines Agency's (EMA) Committee for Advanced Therapies (CAT) and now has up to 6 months to respond. Kiadis expects to fully address the EMA's questions within the agreed upon 6 months and remains on track for a potential regulatory decision in the second half of 2018. If ATIR101 receives conditional or full approval in Europe at this time, this would pave the way for a commercial launch in Europe in 2019.

Expected Upcoming Milestones

- YE 2017- Dose first patient in a Phase III study of ATIR101 in patients with acute hematological malignancy that have received a haplo-HSCT.
- H2 2018- Potential approval (conditional or full) for ATIR101 in the EU.
- 2018- Initiate ATIR101 as adjunctive to PT-Cy and/or other T cell depleted HSCT.
- 2019- Potential commercialization of ATIR101 in the EU.

Analysts

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Market Data

Price	**$9.27**
Market Cap (M)	**$157**
EV (M)	**$144**
Shares Outstanding (M)	**16.9**
Fully Diluted Shares (M)*	**18.1**
Avg Daily Vol	**330,329**
52-week Range:	**$5.97 - $14.51**
Cash (M)	**$47.0**
Net Cash/Share	**$0.72**
Annualized Cash Burn (M)	**$17.0**
Years of Cash Left	**2.8**
Debt (M)	**$35.0**

All relevant values converted at 1 Euro to 1.17 USD
Cash and debt are pro forma
**Does not take into account the exercise of warrants on 10/10/17.*

Financials

FY Dec		2015A	2016A	2017A
EPS	H1	(0.95)A	(0.51)A	(0.63)A
	H2	NA	NA	NA
	FY	(1.45)A	(1.15)A	NA

For analyst certification and disclosures please see page 7

Of note, MolMed's (Milan: MLM.MI) *Zalmoxis* received conditional marketing authorization in the EU in 2016. Their MAA was supported by Phase II data (37 *Zalmoxis*-treated vs. 140 matched controls from the EGMT registry) and included initial results from an ongoing Phase III clinical trial. This may serve as a positive indicator for Kiadis' chances of receiving at least conditional approval based on their Phase II results.

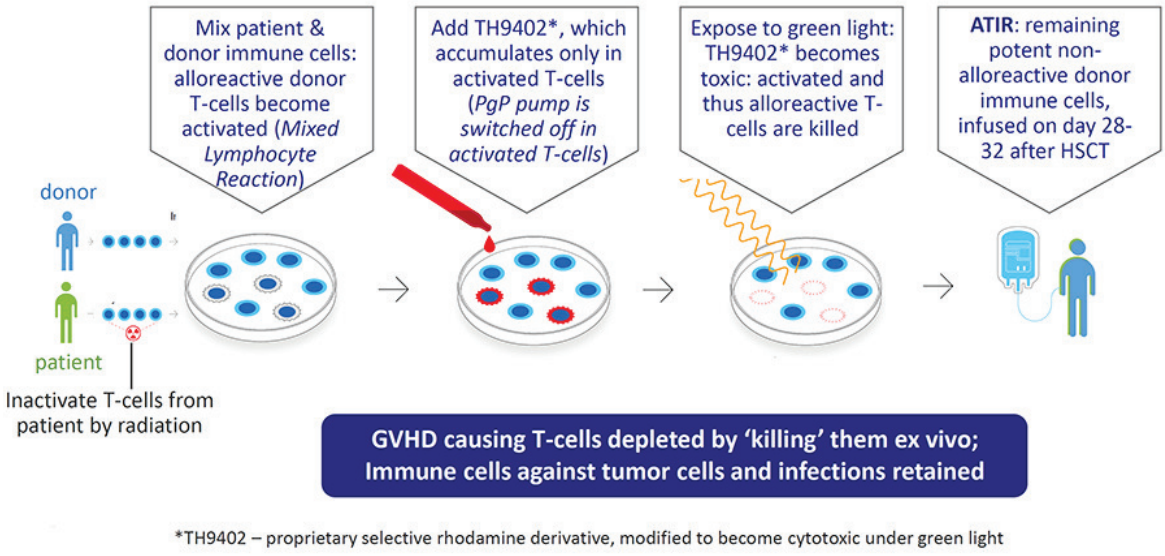
- **FDA RMAT Designation Could Accelerate Approval.** In September, Kiadis announced that it received the Regenerative Medicine Advanced Therapy (RMAT) designation for ATIR101 from the FDA. The RMAT pathway for cell therapies is similar to breakthrough designation for drug candidates, in that it allows Kiadis to interact more frequently with the FDA regarding the clinical testing process. In addition, RMAT designated products may be eligible for priority review, thereby accelerating approval of the therapy. This designation may prove useful as the Company focuses its efforts on filing for the approval of ATIR101 in the US.

- **ATIR101 is a Straightforward Strategy to Make HSCT Safer and More Effective.** Hematological stem cell transplants from half-matched donors have steadily increased in recent years. This is in large part due to the adoption of the post-transplantation cyclophosphamide (PT-Cy) or Baltimore protocol, to deplete in the patient the alloreactive T cells inherent in using a half-matched donor. However, patients undergoing this type of transplant have an increased risk of relapse compared to patients receiving a HSCT from a matched donor, and an even higher risk of developing GvHD. To mitigate this risk, Kiadis is developing ATIR101, an *ex vivo* prophylactic therapy to prevent GvHD, while providing protection against relapse and infection. The therapy is being developed in adult patients with hematological diseases that have received a HSCT from a half-matched donor (i.e. haploidentical donors). The treatment relies on a photoactivatable small molecule that selectively eliminates GvHD causing donor T cells. **Figure 1** depicts ATIR101's mechanism of action. Lymphocytes are harvested from patients prior to conditioning and subsequently inactivated *ex vivo* using radiation. Inactivated lymphocytes from the patient are then mixed with lymphocytes collected from a haploidentical donor. Alloreactivity is triggered when lymphocytes from the donor recognize the patient's lymphocytes as foreign, a process known as a mixed lymphocyte reaction.

  After a four-day incubation, the Company adds 4,5-dibromorhodamine (TH9402), a photosensitizer that enters both rested and activated T cells during the mixed lymphocyte reaction. T cells express P-glycoprotein (Pgp), the protein product of the multi-drug-resistance 1 gene, whose ion transport function is modulated according to the state of T cell activation. Under normal conditions, Pgp extrudes cytotoxic chemicals. However, mitogenic stimulation or MHC-mismatch between cells results in preferential retention of TH9402. After activation with green light at 514 nm wavelength, TH9402 induces oxidative damage within the mitochondria of activated alloreactive T cells, resulting in cell death. After the depletion of alloreactive T cells, donor lymphocytes are frozen in liquid nitrogen, stored, and later infused into the patient 28 to 32 days after a HSCT.

  Our discussions with KOLs highlighted that photodynamic therapy is a well-known and fairly straightforward procedure to deplete alloreactive T cells, with potential to address GvHD within the HSCT space. They highlighted GvHD as an area of high unmet need given that roughly 50% of patients receiving an allo-HSCT will develop some form of GvHD. This high rate underscores the potential use of ATIR101 as an adjuvant therapy across a variety of blood disorders.

Figure 1. Mechanism of Action for ATIR101



Source: Corporate Presentation

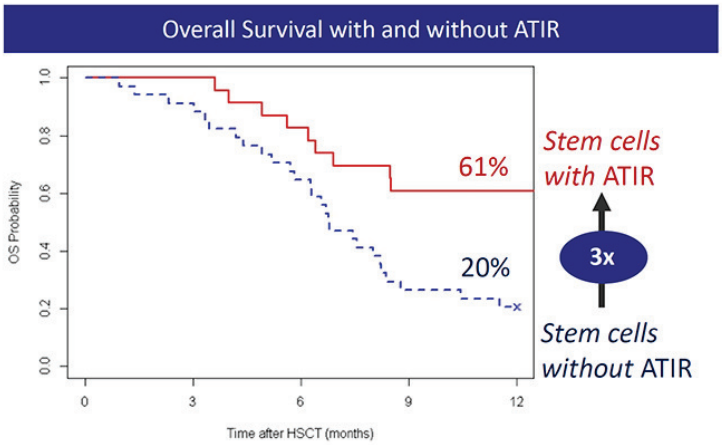
- **Positive Phase II Data with ATIR101 in Acute Blood Malignancies.** Kiadis has reported encouraging data evaluating ATIR101 as an adjuvant therapy for the prevention of GvHD after a haplo-HSCT. Kiadis completed the CR-AIR-007 Phase II trial with ATIR101 and the CR-AIR-006 observational cohort study in acute hematological malignancies. Results from these trials were used to support the Company's MAA submission for ATIR101 in the EU.

  - **CR-AIR-007 Study** – This was a pivotal, open-label Phase II study with ATIR101 in 23 patients with acute myeloid leukemia (AML) or acute lymphocytic leukemia (ALL). Patients received ATIR101 as a donor lymphocyte infusion of 2 x 10^6 $CD3^+$ T cells/kg, 28-32 days after undergoing a T cell depleted $CD34^+$haplo-HSCT from the same donor. The primary endpoint of the trial was transplant-related mortality 6 months after transplantation. Secondary endpoints included overall survival, engraftment, relapse-related mortality, and incidence of acute and chronic GvHD. Patients were followed for two years post treatment.

  - **CR-AIR-006 Observational Cohort Study** – The CR-AIR-006 trial was a historical study that was designed based on scientific advice from the EMA, and followed 34 patients that received a haplo-HSCT between January 2006 and June 2013. Patients were followed for up to 12 months after receiving an HSCT. The efficacy endpoints of the study were transplant-related mortality, relapse-related mortality, overall survival (OS), and progression free survival (PFS).

In order to compare overall survival and the incidence of GvHD between the CR-AIR-007 and CR-AIR-006 studies, patients selected in the CR-AIR-006 study were matched by indication and treatment site to patients in the CR-AIR-007 trial. The twenty-three treated patients in the CR-AIR-007 study were analyzed 12 months post transplantation and compared to patients in the CR-AIR-006 historical cohort study. **Figure 2** shows that 61% of patients in the CR-AIR-007 study, shown in red, were alive a year after transplantation compared to 21% of the patients in the CR-AIR-006 trial, depicted in blue. These results suggest that ATIR101 significantly improves overall survival (OS) compared to patients that had not received ATIR101. However, it should be noted that this study was an open-label trial and did not have a placebo control group.

Figure 2. Overall Survival in Patients Treated with ATIR101 after HSCT

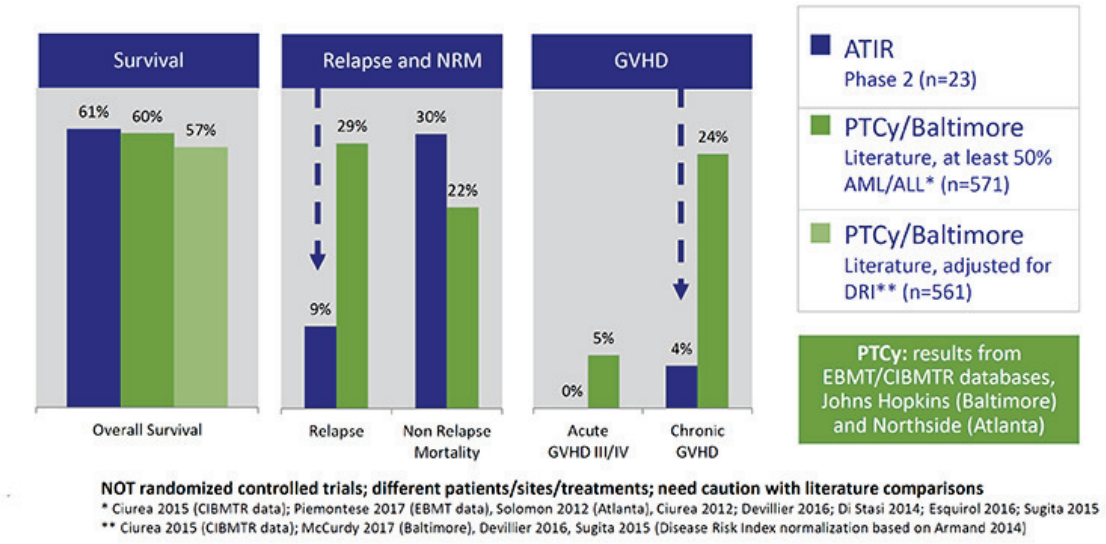


Source: Company Presentation

- **A Retrospective Analysis Evaluating the Efficacy of ATIR101 and PT-Cy.** Kiadis compared results from the CR-AIR-007 study to data from PT-Cy treated patients from various publications. The publications included a historical control group of patients chosen from the European Society for Blood and Marrow Transplantation (EBMT) and the Center for International Blood and Marrow Transplant Research (CIBMTR) databases. The publications also included data from patients treated at Johns Hopkins and at Atlanta's Northside hospital. Patients included in these cohorts were treated with cyclophosphamide after receiving a haplo-HSCT. **Figure 3** shows that 60% of post-transplant cyclophosphamide (PT-Cy) treated patients, shown in green, were alive a year after HSCT. When adjusted for disease risk index (light green), 1 year OS after PT-Cy was 57%. The disease risk index takes into account disease type and disease status at the time of transplantation since they are strong determinants of post-HSCT survival. Although we note the need for caution when comparing data from different clinical trials, these findings suggest that ATIR101, shown in the blue bar, and PT-Cy treatments are comparable in terms of overall survival, which takes into account death due to infections or relapse of malignancy.

  The analysis also evaluated disease relapse, non-transplant related mortality (middle panel) and GvHD (right panel) across each condition. Of note, 9% of patients treated with ATIR101 (blue) experienced disease relapse, which appears to be lower than the 29% rate observed in PT-Cy treated individuals (green). This suggests that ATIR101 may have greater anti-leukemic effects than seen in the historical control group of patients receiving PT-Cy treatment. It also implies that the donor T cells that are infused into the patient, are both viable and able to exert these effects. Importantly, none of the ATIR101 treated patients showed grade III/IV acute GvHD, which is lower than the 5% rate observed in the historical control group, suggesting that ATIR101 may offer better protection against acute GvHD than PT-Cy. This retrospective analysis also evaluated chronic GvHD in both groups. Analysis of the data showed a 4% rate of chronic GvHD among ATIR101 treated patients, which was less than the 24% rate found in the historical control cohort.

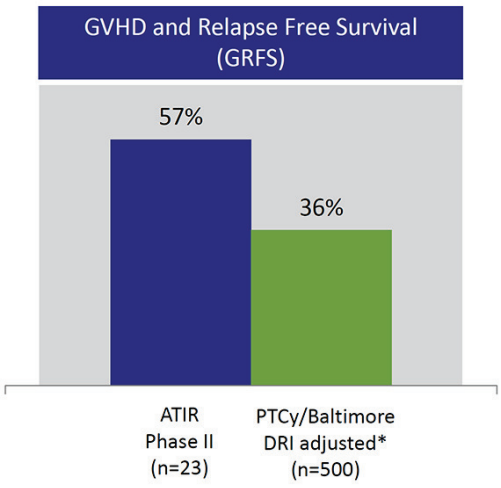
Figure 3. Overall Survival, GvHD, Relapse Rates in ATIR Treated Patients



Source: Company Presentation

- **ATIR101 Improves Measures of GvHD and Relapse-Free Survival.** The Company also evaluated GvHD and relapse free survival (GRFS) in ATIR101 treated patients and compared these values to GRFS rates from two studies (Solh et. al. 2016 and McCurdy et. al. 2015) where patients were treated with PT-Cy following a haplo-HSCT. GRFS is a composite measurement that takes into account survival without evidence of disease relapse, chronic GvHD requiring immunosuppression, or acute grade III/IV GvHD. **Figure 4** shows data comparing patients treated with ATIR101 in the CR-AIR-007 study to a disease risk normalized literature cohort of patients that had undergone PT-Cy treatment after receiving a haplo-HSCT. A retrospective analysis of the CR-AIR-007 study found that 57% of ATIR101 treated patients experienced GRFS 1 year after receiving a HSCT. The same analysis found that 36% of patients in the literature cohort experienced GRFS 1 year after receiving a HSCT. This suggests that ATIR101 protects patients from both GvHD and disease relapse to a greater degree than PT-Cy. This is important since GRFS may be a better measure of health after receiving a HSCT, and will be used as the primary endpoint in the Company's announced Phase III trial.

Figure 4. GvHD and Relapse Free Survival (GRFS) in ATIR Treated Patients



Source: Company Presentation

- **Phase III Trial with ATIR101 in Acute Hematological Malignancies.** Kiadis announced that it will start a randomized controlled Phase III study with ATIR101 in patients with hematological malignancies including AML, myelodysplastic syndromes (MDS), and ALL. The Company expects to enroll 195 patients to be randomized to receive either PT-Cy treatment or a single dose of ATIR101. Patients in the PT-Cy arm of the study will receive a T cell replete haplo-HSCT, followed by 50 mg/kg/day of cyclophosphamide at day 3 and at day 4/5 after the HSCT. Patients in the ATIR101 arm will receive a T cell depleted $CD34^+$ haplo-HSCT, followed by ATIR101 as a donor T cell infusion at 2.0 $x10^6$ T cells/kg, 30 days after HSCT. This is the same protocol that was used in the completed Phase II study. The primary endpoint of the Phase III trial is GRFS. Key secondary endpoints include overall survival and relapse-related mortality. Recall that GRFS is a composite measurement that takes into account survival without relapse, grade III-IV acute GvHD, or chronic GvHD that requires use of immunosuppressants. The trial is designed to detect an absolute 20% difference in GRFS and a primary analysis will occur after 93 GRFS events. In addition to clinical efficacy, the Company will also collect pharmacoeconomic data regarding cost, length of hospital stays, incidence of infection, and quality of life.

  The announced Phase III study will be performed in the US, Canada, and Europe, and will begin enrollment this quarter. Kiadis expects full enrollment to take between roughly a year and a half to two years. It expects for topline results one year after full enrollment, although this depends on the rate in which the trial accumulates GRFS events.

- **New CMO with a Track Record of Launching Oncology and Cell Therapies.** Kiadis recently announced the appointment of Dr. Andrew Sandler as the new Chief Medical Officer (CMO) of the Company. Before joining Kiadis, Dr. Sandler was the senior vice president of medical affairs at Medivation, which is now a Pfizer (NYSE: PFE) company. Prior to that, he was the CMO at Dendreon Pharmaceuticals and Spectrum Pharmaceuticals (NasdaqGS: SPPI), and also held senior level positions at Bayer Healthcare and Seattle Genetics (NasdaqGS: SGEN). Dr. Sandler has over 20 years of industry experience in the oncology space. His appointment provides additional experience to the management team as they focus on developing and commercializing ATIR101 in the EU and US.

- **Market Opportunity for ATIR101 in Hematological Malignancies.** Kiadis is developing ATIR101 to prevent GvHD and mitigate the risk of disease relapse and infection in patients undergoing allogeneic HSCT (allo-HSCT). In 2015, roughly 8,000 allo-HSCT were reported in the US and 17,000 in the EU. The increase in allo-HSCT in recent years has been driven in part by a growth in haplo-HSCT. ATIR101 is intended for adult patients with acute hematological malignancies undergoing allo-HSCT, which makes up 80% of the transplantations performed. In addition, Kiadis' therapy could open up the use of haplo-HSCT to patients that have yet to find a donor. As many as 35% of those who need to receive a HSCT fail to find a donor. We estimate that this expands Kiadis' potential market to 11,000 patients in the US and roughly 23,000 in the EU. If the product is ultimately approved, this could translate into sales of up to $600 million in the US and $900 million in the EU at peak penetration.

- **ATIR101 Complements Current Transplant Procedures.** ATIR101 can be seamlessly integrated into routine HSCT transplantation schemes. The proposed manufacturing process for ATIR101 involves the apheresis of cells from donors and patients 14 days prior to the HSCT conditioning regimen. The harvested cells are transported to Kiadis' facility and processed using the Company's proprietary photodynamic therapy. Once the material is processed, the ATIR101 therapy is frozen in liquid nitrogen and shipped to the hospital where it is later dosed into a patient after receiving a HSCT. The low cost of goods involved in Kiadis' approach could lead to higher margins for the Company compared to therapies that employ genetic modification of donor cells.

Risk to Invesment

We consider an investment in Kiadis to be a high-risk investment. Kiadis is a developmental stage company with no history of taking a treatment to market, and currently has no FDA or EMA approved products in its portfolio. The Company's products in development may fail in clinical trials or fail to be approved by the FDA or other regulatory agencies. Furthermore, early indications of efficacy do not necessarily translate into positive late-stage results. As with any company, Kiadis may be unable to obtain sufficient capital to fund planned development programs. Regulatory approval to market and sell a drug does not guarantee that the drug will penetrate the market, and sales may not meet the expectations of investors.

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