Annual Results 2017 & Business Update

13 April 2018

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HIGHLIGHTS - Operational

- Filed Marketing Authorization Application with the European Medicines Agency for ATIR101 in blood cancers
- Submitted responses to the EMA to enable a conditional marketing approval from the European Commission - potentially allowing an opinion from the EMA in Q4 2018
- Received Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. FDA for ATIR101
- First patient enrolled in Phase 3 trial for ATIR101 in adult patients with blood cancer
- Leased existing commercial manufacturing facility in The Netherlands
- Strengthened Organization and Supervisory Board

HIGHLIGHTS – Financial

- Raised more than EUR 60 million in equity and debt since June 2017
- End of March 2018: EUR 47.7 million cash

(Amounts in EUR million, except per share data)	2017	2016	Change
Total revenue and other income	**-**	**-**	**-**
Total operating expenses	**(16.1)**	**(11.4)**	**(4.7)**
Research and development	(11.2)	(8.2)	(3.0)
General and administrative	(4.9)	(3.2)	(1.7)
Operating result	**(16.1)**	**(11.4)**	**(4.7)**
Net financial result	**(0.9)**	**(3.4)**	**2.5**
Net result	**(17.0)**	**(14.8)**	**(2.2)**
Net operating cash flow	**(15.9)**	**(14.3)**	**(1.6)**
Cash position at end of year	**29.9**	**14.6**	**15.3**
Equity	**15.9**	**9.4**	**6.5**
Earnings per share before dilution (EUR)	**(1.14)**	**(1.08)**	**(0.06)**

ATIR Regulatory Status

Product	Pre-Clinical	Phase I	Phase II	Phase III	Filing	Catalysts	Commercial Rights
ATIR101 (Europe)	*Orphan Drug Designation*					- CHMP Opinion 4Q18 - EU Launch 2H19	Kiadis pharma
ATIR101 (USA)	*Orphan Drug & RMAT Designations*					- Phase III (interim) read out	Kiadis pharma

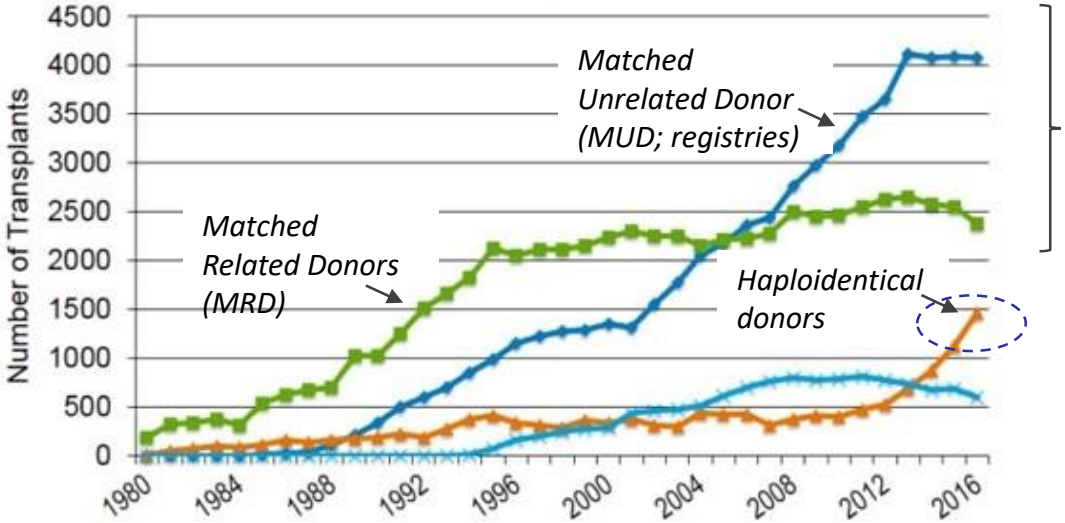
The very first cell transplant method: allogeneic HSCT

Allogeneic Hematopoietic Stem Cell Transplantation (HSCT):

- **Curative intent**: replace disease blood/immune system with healthy one from donor
- **Risk of Graft versus Host disease (GVHD)**: Donor immune system attacks the patient
- **Mostly blood cancers** (85%) **and adults** (82%)

Adoption of HSCT limited by high risk	**Blood cancers: Only 20-30% long term GVHD-Free and Relapse-Free Survival (GRFS)**
	Inherited blood disorders or autoimmune disease: Risk of replacing chronic disease with (chronic) GVHD

HSCT: Strong growth, still large unmet need (U.S.)



Unmet need: 13,000 per year (lack of matched donors)

<u>Historical</u>: Matched Related or Unrelated Donors

- Donor availability 20-80% (due to family size & genetic diversity)
- Declining, despite unmet need

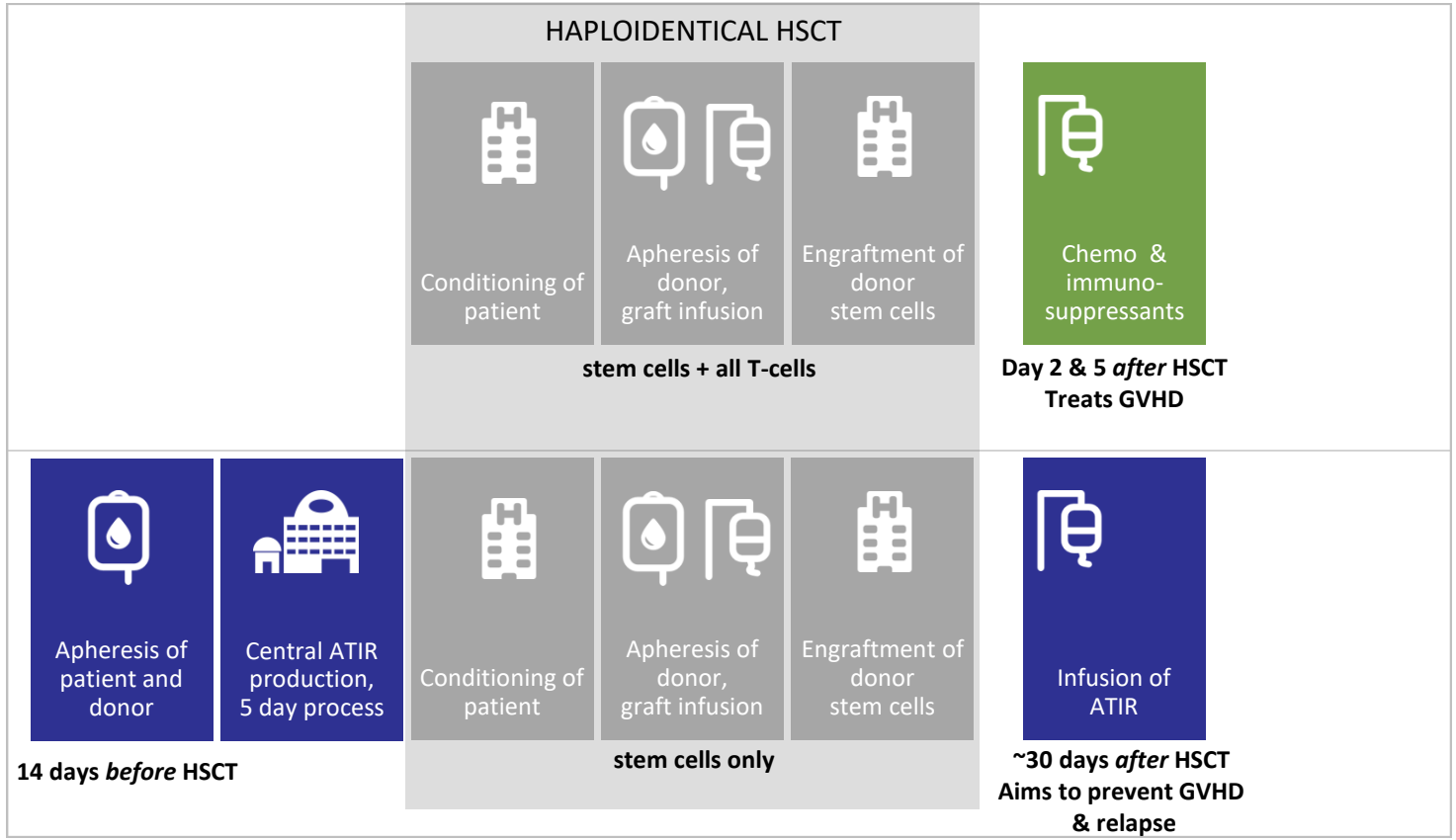
<u>Emerging</u>: Haploidentical or half matched donors

- Donor availability >95% (parents/children)
- 32% compound annual growth
- Made possible due to **P**ost **T**ransplant **Cy**clophosphamide (PTCy) or 'Baltimore' protocol*

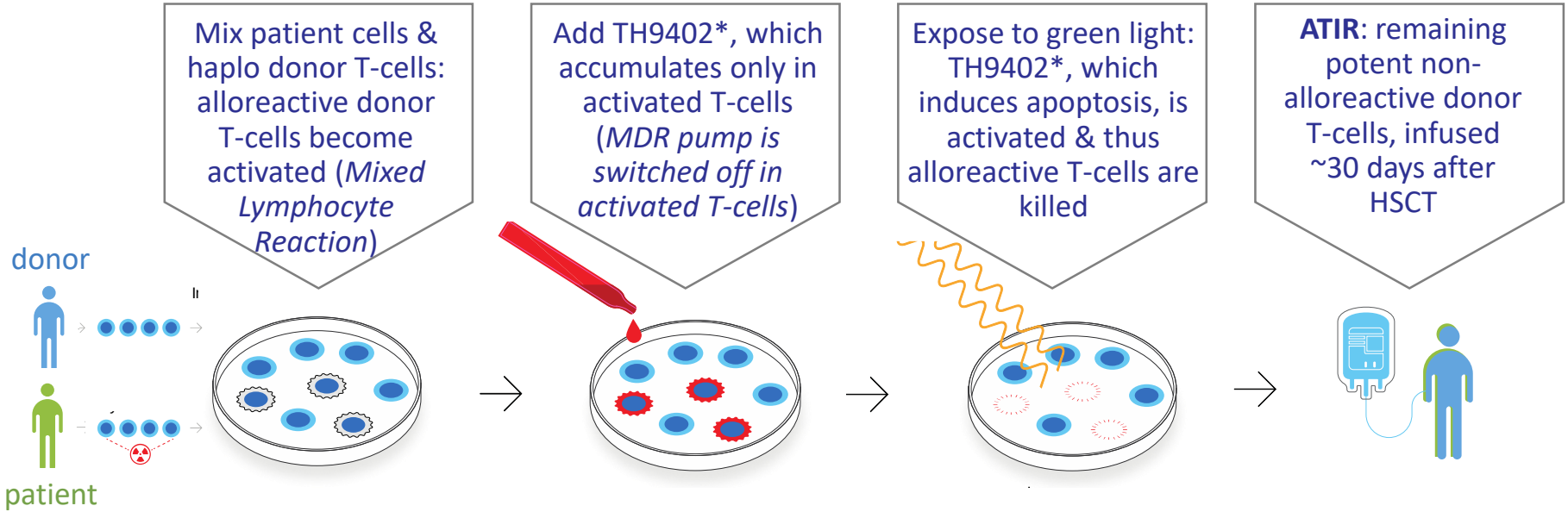
Source: CIBMTR 2017 Summary slides; Fuchs 2017; Gragert 2014; Besse 2015

* Cyclophosphamide (chemotherapy, days 3 and 5) & immunosuppressants to treat immediate attack from alloreactive haploidentical donor T-cells

Kiadis: potential improvement vs. PTCy/Baltimore



ATIR production: subset of T-cells that protect, but not attack

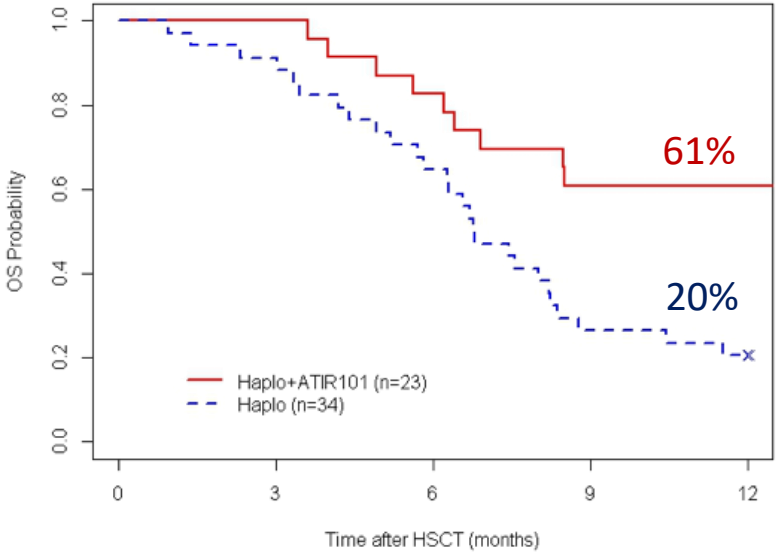


Protect: Retain protective T-cells to fight relapse and infections
& Not attack: Reduce risk of GVHD by depleting alloreactive T-cells *ex vivo*

*TH9402 – proprietary selective rhodamine derivative, modified to become cytotoxic under green light

Phase 2 (007): potent T-cell product, yet low GVHD (1 yr)

Improved Overall Survival due to ATIR



CD34+ stem cells *with* ATIR

3x

CD34+ stem cells *without* ATIR

007: Haplo CD34+ plus single dose ATIR

- Open label single arm 2013-18
- 23 AML/ALL patients receiving ATIR (MITT)
- 4 sites Canada/EU
- Dose 2 million cells/kg*

006: Haplo CD34+

- Historical observational cohort 2006-13
- 35 patients, similar indications/sites
- Protocol based on EMA scientific advice

Low GVHD due to ATIR

- **no** acute grade III/IV
- 3 acute grade II **(13%)**
- 1 chronic **(4%)**

2 million cells/kg of potent T-cells: increasing survival 3x, yet low GVHD**

No need for prophylactic immunosuppression

* Non allodepleted donor lymphocyte infusion can cause severe GVHD at 10,000 cells/kg

Phase 2 (007): relapse, GVHD & GRFS* vs. literature for PTCy

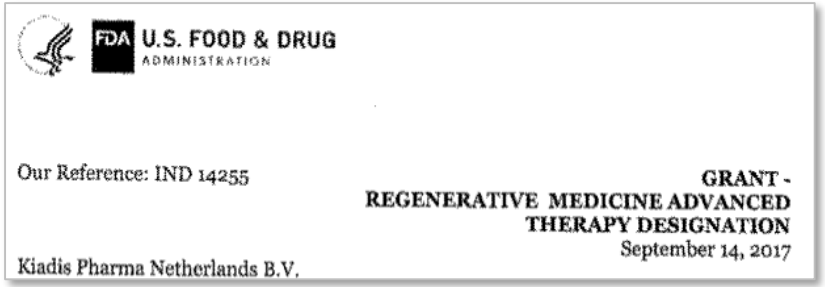
Endpoint at 12 months	ATIR101 as adjunct to haploidentical T-cell depleted HSCT (CR-AIR-007 study in acute leukemia; 23 patients)	PTCy / Baltimore protocol with haploidentical T-cell replete HSCT (available literature)**
Relapse rate	9%	29%
Chronic GVHD rate	4%	24%
Grade III/IV acute GVHD rate	0%	5%
GVHD-Free and Relapse-Free Survival	57%	36%***

Comparison provided for illustrative purposes, based on literature comparison, NOT based on randomized controlled trials

* Defined as survival without chronic GVHD requiring immunosuppression, acute grade III/IV GVHD or relapse
** Ciurea 2015; Piemontese 2017, Solomon 2012, Ciurea 2012; Devillier 2016; Di Stasi 2014; Esquirol 2016; Sugita 2015
*** Solh 2016 (Atlanta; DRI normalized GRFS 30%; n=128); McCurdy 2017 (Johns Hopkins; DRI normalized GRFS 38%; n=372)

Filed in EU & received ‘breakthrough’ in US, based on Phase 2

EMA (EU)	**Marketing Authorization Application filed, potential (conditional) opinion Q4 2018**	• ATMP certificate for quality and non-clinical data in 2015 • Pediatric Investigation Plan agreed • Phase 2 and historical control accepted for filing and review* • Day 120 questions submitted end Q1 2018
FDA (U.S.)	**Regenerative Medicine Advanced Therapy designation received (same benefits as Breakthrough)**	• Increased access to FDA (not limited to customary timepoints) • Possibility for priority/rolling review of BLA • Development support (program/endpoints)


FDA U.S. FOOD & DRUG ADMINISTRATION

Our Reference: IND 14255

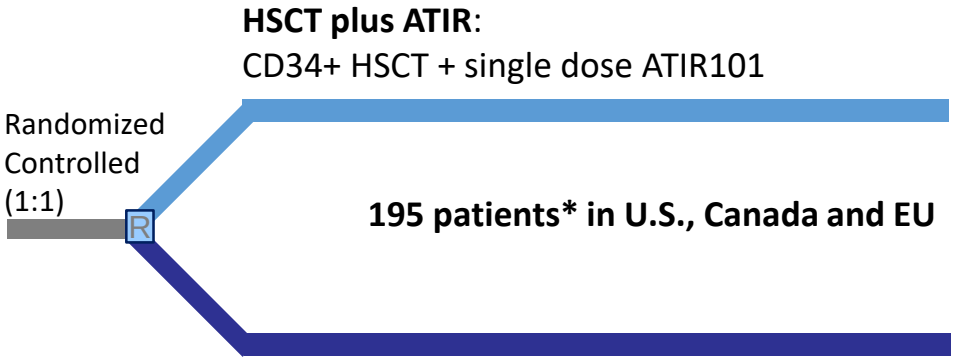
GRANT -
REGENERATIVE MEDICINE ADVANCED THERAPY DESIGNATION
September 14, 2017

Kiadis Pharma Netherlands B.V.

* Various hemato-oncology products (conditionally) approved by EMA based on Phase 2, e.g., Zalmoxis (MolMed, 36 patients, versus matched historical control), Blincyto, Venclexta, Bosulif

Commenced Phase 3 Clinical Study

Objectives: demonstrate superior clinical benefit and collect pharmacoeconomical data (cost, days in hospital, incidence of severe infections and quality of life)



Primary endpoint:
GVHD-Free and Relapse-Free Survival (GRFS**)

Aligned with FDA and regulators in EU; Enrolling patients

* 80% powered to detect 20% GRFS difference; 15% difference will be statistically significant; allowed under protocol to increase sample size; 245 pts would give 80% power to detect 18% difference
** Survival without chronic GVHD requiring immunosuppression, acute grade III/IV GVHD or relapse

Kiadis key milestones and upcoming catalysts

2017	EMA submission of ATIR for marketing authorization approval	✔
	First patient enrolled for ATIR Phase 3	✔
	Updates enrollment, regulatory, new clinical sites	✔
	FDA Regenerative Medicine Advanced Therapy designation	✔
	Secured own commercial manufacturing facility (lease)	✔
	New management and supervisory board members	✔
2018	Completion of enrollment of second Phase 2 trial (CR-AIR-008)	✔
	Submission of answers to EMA Day 120 questions (End Q1)	✔
	Potential EU CHMP opinion, Q4	
	Updates Phase 2 data and Phase 3 enrollment	
2019	Potential initial commercial launch ATIR in first of EU5 countries (H2)	
	Initiate trial with ATIR as adjunctive to PTCy	
	Potential interim read out Phase 3	