

## Current Pipeline & Development Status

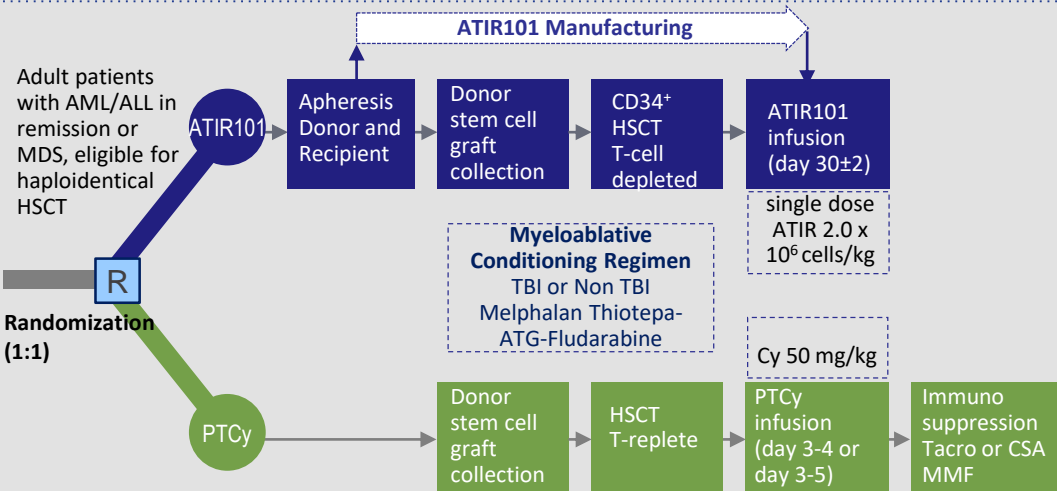
Region	Phase I	Phase II	Phase III	Filing	Catalysts	Commercial Rights	Comments
EU	Orphan Drug Designation				• EU Approval '20	Kiadis Pharma	Procedure ongoing
US	Orphan Drug & RMAT Designations				• Phase 3 interim read out ('21; 105 events)	Kiadis Pharma	RMAT 'breakthrough' designation (9/2017; FDA access, priority review, support)

All trials: Patients: adult AML/ALL/MDS; Conditioning: Myeloablative; Graft source: Haplo PBMCs; HSCT: T-cell depleted (CD34+); ATIR: ~30 days after HSCT; No prophylactic immunosuppression

### Phase 3: CR-AIR-009; Single dose ATIR

**OBJECTIVE:** Demonstrate superior clinical benefit and collect pharmacoeconomic data

Randomized/controlled (ATIR versus PTCy) • Initiated 2017 (enrolling) • 250 patients • Event driven primary endpoint (GRFS) • ~50 sites planned CA, Europe, US, Israel



**PRIMARY ENDPOINT:**

GVHD-Free & Relapse-Free Survival (GRFS): survival without relapse, grade III/IV acute GVHD or chronic GVHD requiring immunosuppression

- Interim analysis: at 2/3 of events (17,6% GFRS treatment effect, hazard ratio 0.61)
- Primary analysis: at 156 events (11,4% GFRS treatment effect, hazard ratio 0.73)

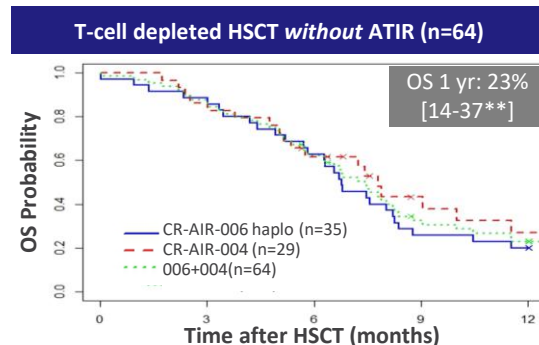
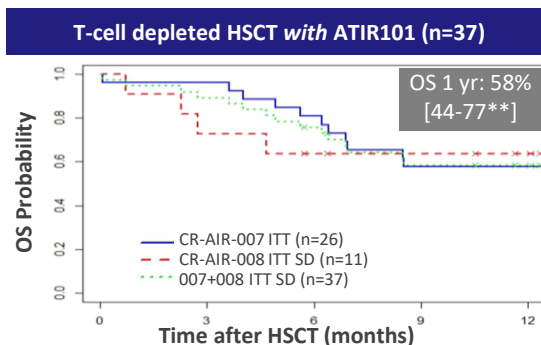
**SECONDARY ENDPOINTS:**

Overall Survival (OS), Progression Free Survival (PFS), Relapse, Non Relapse Mortality (NRM)

**OTHER:**

Randomized at enrollment; Balanced conditioning regimens in ATIR/PTCy arms; Stratification for Disease Risk Index, underlying disease and treatment site

### Phase 2: CR-AIR-007/008/006/004; Improved Overall Survival (ITT\*, Single Dose; 1 yr) P=0.005



\*ITT (intention to treat): all patients undergoing T-cell depleted CD34+ HSCT; SD: single dose; CR-AIR-008 status 1 June 2018 (3 patients at risk); \*\* 95% confidence interval

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## Phase 2 Studies in AML/ALL/MDS

### CR-AIR-007: Single dose ATIR

- Open label single arm; 2013-2018
- 23/26 patients (MITT/ITT\*)
- 2 year follow up
- Sites in BE, CA, GE, UK

### CR-AIR-008: Single dose ATIR and two doses ATIR\*\*

- Open label single arm; 2015-2018 (ongoing)
- 9/11 single dose; 6/6 patients two doses (MITT/ITT)
- 3 patients still at risk
- 1 year follow up
- Sites in CA, BE, GE, UK

### CR-AIR-004: Historical Control, No ATIR

- Open label single arm; 2009-2012
- 40 patients (29 matched to CR-AIR-007)
- Sites in BE, CA, GE, NL, UK, US

### CR-AIR-006: Historical Control, No ATIR

- Observational cohort, EBMT registry; 2006-2013
- 35 patients (all matched to CR-AIR-007)
- 1 year follow up

\* MITT: Modified Intent to Treat (transplanted and ATIR); ITT: Intent to Treat (transplanted); \*\* CR-AIR-008 was designed to test safety of second dose, but due to higher than expected GVHD it was decided to stop infusing second dose (in accordance with protocol)

ATIR Phase 2 Data	T-cell depleted (CD34+) with single dose ATIR (n=37)*			T-cell depleted (CD34+) without ATIR (n=64)*		
	CR-AIR-007	CR-AIR-008	007-008	CR-AIR-006	CR-AIR-004	004-006
<b>1 year post HSCT</b>	<b>(ITT n=26)</b>	<b>(ITT n=11)</b>	<b>(ITT n=37)</b>	<b>(n=35)</b>	<b>(n=29)</b>	<b>(n=64)</b>
Overall Survival	58% (42-80)	64% (41-100)	58% (44-77)	20% (10-39)	27% (13-54)	23% (14-37)
Non-relapse mortality	35%	27%	33%	66%	59%	63%
Relapse-related mortality	8%	9%	8%	15%	14%	14%
Relapse	8%	9%	8%	NA	NA	NA
Acute GVHD grade II-IV	19%	27%	21%	20%	18%	19%
Acute GVHD grade III-IV	0%	18%	5%	6%	7%	6%
Chronic GVHD	4%	0%	3%	11%	5%	8%
Chronic GVHD severe	0%	0%	0%	9%	5%	7%
<b>6mths post HSCT</b>	<b>(MITT n=23)</b>	<b>(MITT n=9)</b>	<b>(MITT n=32)</b>	<b>(n=35)</b>	<b>(n=29)</b>	<b>(n=64)</b>
Non-relapse mortality	13% (0-27)	11% (0-29)	13% (0-24)	37% (19-51)	35% (15-51)	36% (23-47)

\*Notes: NRM at 6 months primary endpoint of CR-AIR-007; Kaplan-Meier estimates for OS and NRM at 6 months; all other estimates cumulative incidence analyses; ITT: patients receiving HSCT; MITT: patients receiving HSCT and ATIR; OS: overall survival; CR-AIR-008 status 1 June 2018 (3 patients at risk); All trials: Conditioning: Myeloablative; Graft source: Haplo PBMCS; HSCT: T-cell depleted; ATIR: ~30 days after HSCT; No prophylactic immunosuppression

## ATIR101 Compared to PTCy Literature Studies (basis for phase 3 design)

### 1-year Patient Outcomes after T-cell Depleted Haplo-HSCT with ATIR101 or T-Cell Replete HSCT with PTCy

Average	ATIR101 (Two phase 2 trials: N=37; ITT)		PTCy <sup>λ</sup>
Relapse	8%	25%	N=2579 <sup>5,8,10-32</sup>
RRM	8%	14% <sup>Y</sup>	N=2384 <sup>5,8,10-29,31-33</sup>
NRM	33%	22%	N=1996 <sup>5,10-29,31-33</sup>
aGVHD (grade III/IV)	5% <sup>*</sup>	8% <sup>ξ</sup>	N=1419 <sup>5,10-13,16-22,25-30</sup>
cGVHD	3%	22%	N=1312 <sup>5,10,12-14,16-22,24-27,29,31</sup>
OS	58%	64%	N=2384 <sup>5,8,10-29,31-33</sup>

\*All grade III aGVHD. †Data are weighted by number of patients in each of the PTCy publications with >50% AML/MDS/ALL. ‡Calculated as RRM = (100 - OS) - NRM; note that the N-value for NRM was lower (N=1996) than for OS (N=2384). §Day 100 grade III/IV aGVHD.

### 1-year GRFS after T-cell Depleted Haplo-HSCT with ATIR101 or T-Cell Replete Haplo-HSCT with PTCy

Average	ATIR101	PTCy studies	
	Two phase 2 trials (N=37)	Johns Hopkins (N=372)	Northside (N=128)
DRI	L: 0% I: 57% H: 43%	L: 14% I: 67% H: 19%	L: 19% I: 39% H: 40%
1 yr GRFS (95% CI)	53% (39-72) <sup>†</sup>	45% (40-50)	33% (25-41)
DRI adjusted 1 yr GRFS <sup>α</sup>	53% (39-72) <sup>†</sup>	40%	30%

\*Kaplan-Meier estimate of 1-year GRFS in 37 patients in the ATIR101 studies; <sup>†</sup>Normalized according to the DRI profile in the ATIR101 clinical trials population to allow comparison.; H, high and very high; I, intermediate; L, low

PTCy Literature References: 5. Ciurea SO, et al. Blood 2015; 126: 1033-1040; 8. McCurdy SR, et al. Haematologica 2017; 102:391-400; 10. Devillier R, et al. ASBMT Abstracts 2015; 6:8388-8396; 11. Di Stasi A, et al. Biol Blood Marrow Transplant 2014; 20:1975-1981; 12. Ciurea SO, et al. Biol Blood Marrow Transplant 2012; 18:1835-1844; 13. Solomon SR, et al. Biol Blood Marrow Transplant 2012; 18:1859-1866; 14. Esquirol A, et al. Bone Marrow Transplant 2016; 51:S314-S513; 15. Piemontese S, et al. J Hematol Oncol 2017; 10:24; 16. Sugita J, et al. Biol Blood Marrow Transplant 2015; 21:1646-1652; 17. Kanate AS, et al. Blood 2016; 127:938-947; 18. Bashey A, et al. Biol Blood Marrow Transplant 2016; 22:125-133; 19. Slade M, et al. Biol Blood Marrow Transplant 2017; 23:1736-1743; 20. Kasamon YL, et al. Blood Adv 2017; 1:288-292; 21. Fraccaroli A, et al. Am J Hematol 2018; Epub ahead of print; 22. Baker M, et al. Biol Blood Marrow Transplant 2016; 22:2047-2055; 23. Carnevale-Schianca F, et al. Biol Blood Marrow Transplant 2017; 23:459-466; 24. Kwon M, et al. Bone Marrow Transplant 2017; 52:1138-1143; 25. Ruggeri A, et al. Haematologica 2017; 102:401-410; 26. Srour SA, et al. Biol Blood Marrow Transplant 2017; 23:318-324; 27. Devillier R, et al. Bone Marrow Transplant 2016; 51:194-198; 28. Duléry R, et al. Biol Blood Marrow Transplant 2018; 24:1013-1021; 29. Gaballa S, et al. Cancer 2016; 122:3316-3326; 30. Lorentino F, et al. Blood Adv 2017; 1:669-680; 31. Rashidi A, et al. Bone Marrow Transplant 2016; 51:1561-1564; 32. Robin M, et al. Biol Blood Marrow Transplant 2018; Epub ahead of print; 33. Robin M, et al. Blood Adv 2017; 1:1876-1883; 34. Santoro N, et al. J Hematol Oncol 2017; 10:113; 35. Rovers J, et al. EBMT 2017; poster presentation #A171; 36. Clinicaltrials.gov. Available at: <https://clinicaltrials.gov/ct2/show/NCT02999854> (accessed November 2018).