



SAVING LIVES WITH INNOVATIVE CELL-BASED THERAPY

Cell-based immunotherapy products for the treatment of blood cancers and inherited blood disorders

Company Presentation

AGM, 28 June 2016

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Company highlights 2015/2016 & intro to ATIR

1

*Positive primary endpoint data announced at EBMT conference in April 2016
Positive Phase II interim data announced at ASH conference in December 2015*

2

*Double dosing Phase II study with ATIR101 enrolling and treating patients
Certificate received from EMA for quality and non-clinical data
Rapporteurs appointed by EMA for MAA pathway*

3

*Closed manufacturing process established and to be used in Phase III
Technology transfer to manufacturing sites for Phase III w/o complications so far*

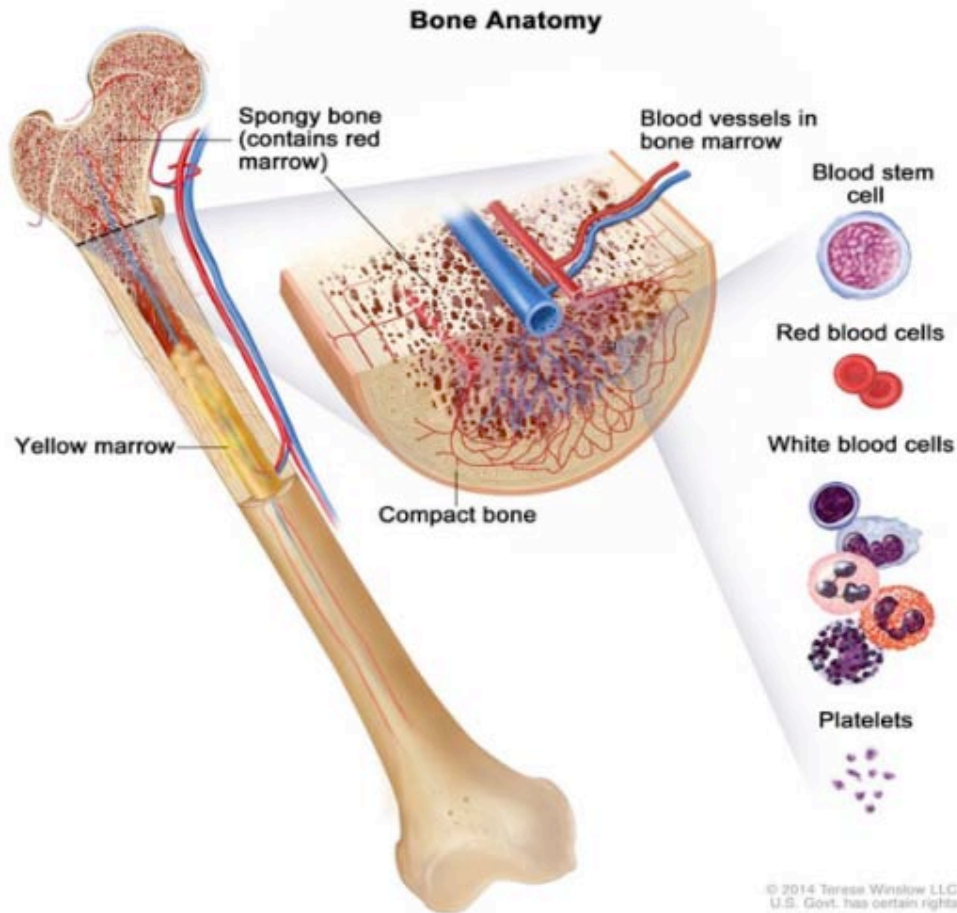
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*Partnered with Leukemia and Lymphoma Society, US
Collaboration with Thalassaemia International Federation*

5

Successful IPO on Euronext in Amsterdam and Brussels

HSCT as curative treatment for blood cancer and inherited blood disorders

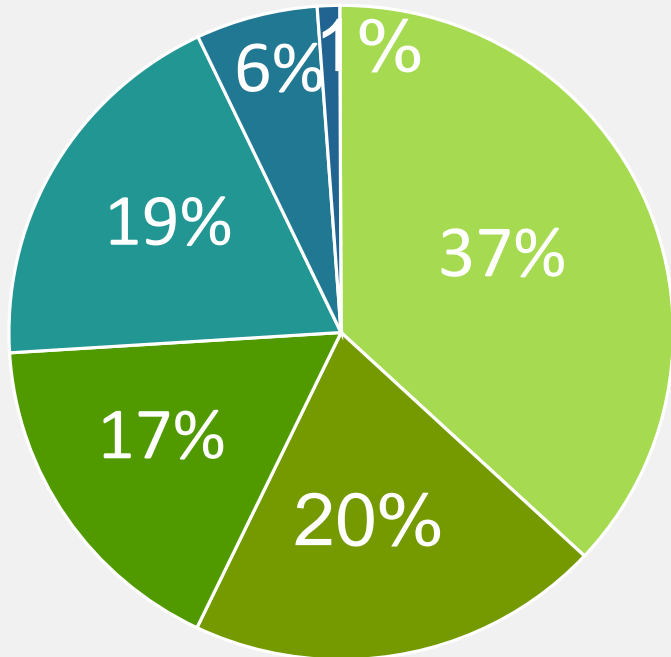


Basic principle of HSCT

1. Destroy the diseased bone marrow
2. Create bone marrow space in the recipient for the donor cells to engraft
3. Replace it with healthy donor cells
4. Suppress the immune system or eliminate the recipient T-cells to minimize risks of rejection

Significant risks & limitations in HSCT

Causes of death after MUD HSCT



CIBMTR-data includes centers located globally and is measured over 2011-2012

Risks

Opportunistic infections

- Immunosuppressed patients are highly susceptible to pathogens

Graft-versus-Host Disease (GVHD)

- GVHD occurs when donor T-cells recognize a patient's tissue as 'foreign'. Incidence of grade III/IV GVHD is as high as 30% and often fatal

Limitations

Cancer relapse

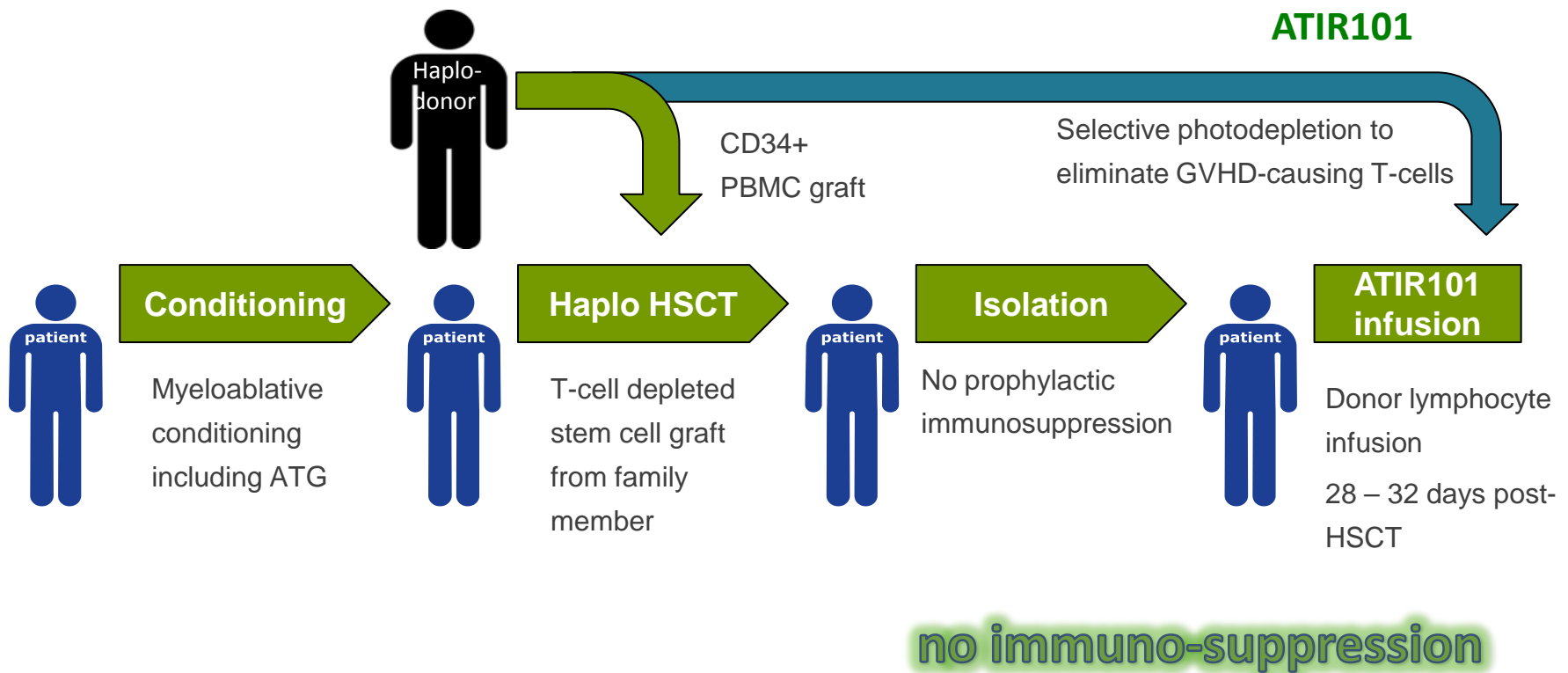
- Ablated immune system has poor ability to fight residual leukemic cells

Donor availability

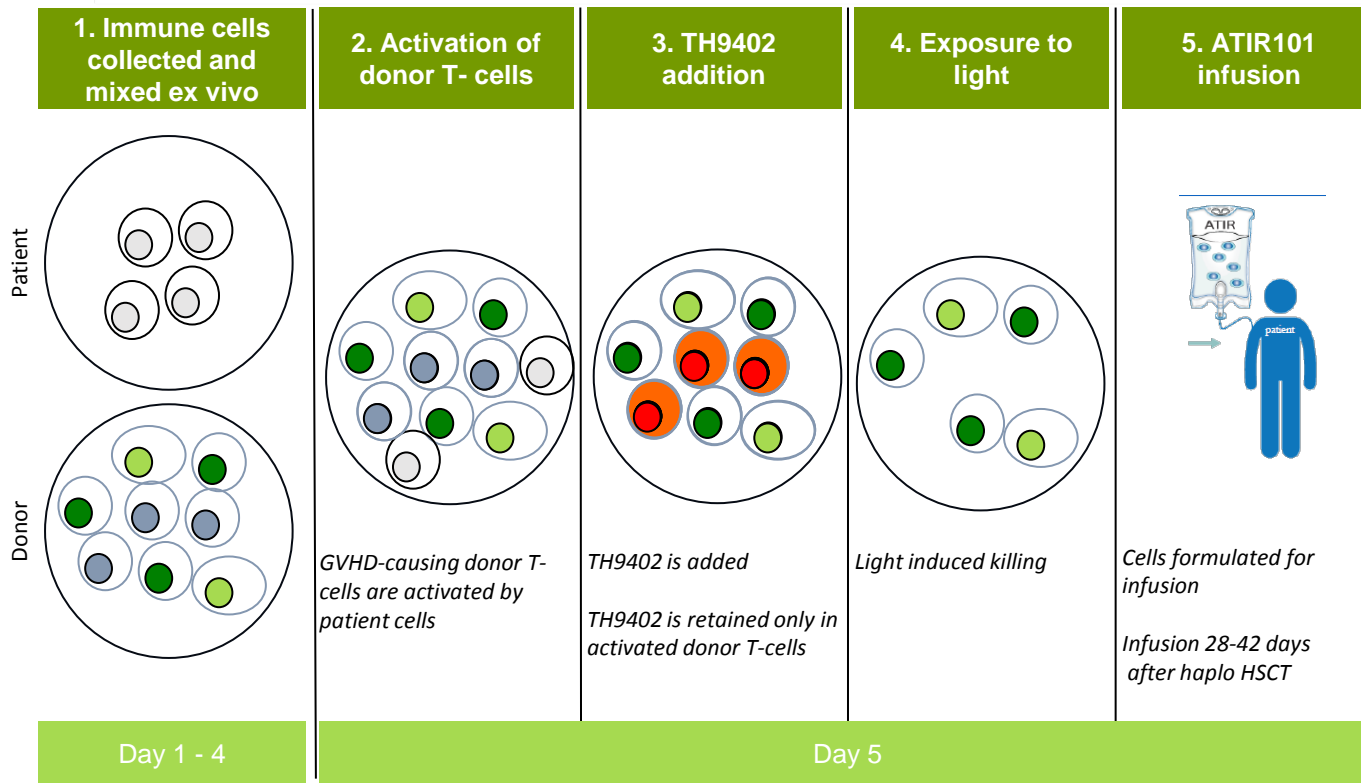
- Approx. 75% of patients do not have a matched family donor and approx. 35% of patients eligible for HSCT will not find a matched donor in time

Haplo-HSCT with ATIR101: addressing risks and limitations of HSCT within standard hospital procedures

- ATIR101 allows for an immunosuppressant-free transplant regimen for haploidentical donor transplantation (i.e. from a family member)
- Adjunctive to (partially) T-cell depleted stem cell graft



ATIR101 manufacturing removes GVHD-causing T-cells while retaining key immune cells



ATIR101 characteristics

- Selective removal of GVHD-causing T-cells
- Key immune cells are retained to protect against infections
- T-cells directed against leukemic cells are retained



Non-activated GVHD-causing donor T-cells



Activated GVHD-causing donor T-cells



Activated GVHD-causing donor T-cells with TH9402 retained



Irradiated patient cells



Graft-versus-Leukemia T-cells

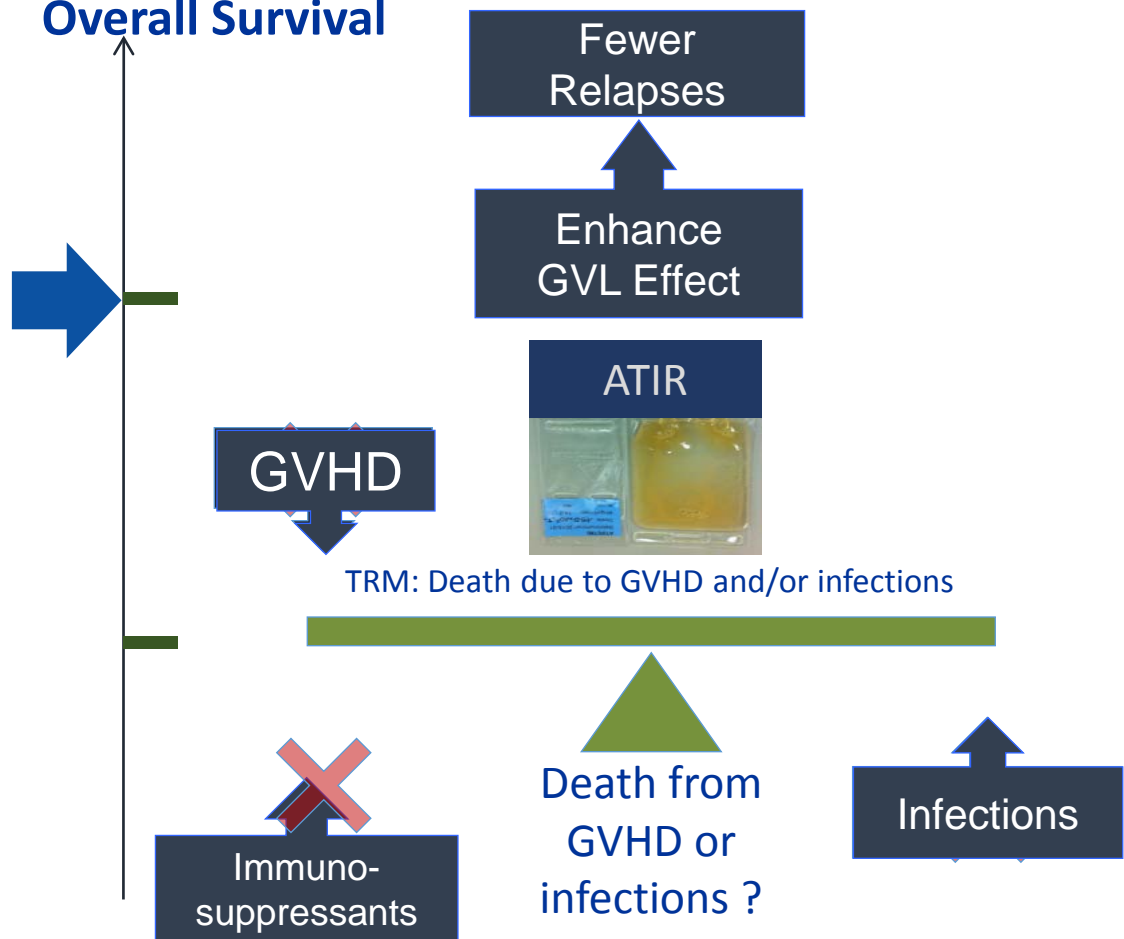


Other immune cells (e.g. virus specific memory and effector cells, naïve cells, NK cells)

ATIR101: low/no GVHD, low infections, low relapse and high overall survival

ATIR101 benefits

Overall Survival



Remarks

Infusing more donor T-cells after the HSCT can provide better/faster immune protection, but:

- GVHD more likely to occur
- Immuno-suppression required to control GVHD
- Risk of infections increasing
- ATIR101 has not been shown to elicit life-threatening GVHD
- With ATIR101 immuno-suppression is not needed
- ATIR101 has been shown to reduce the risk of life-threatening infections
- ATIR101 has been found to provide Graft-versus-Leukemia (GVL) effect

Overview of ATIR101/201 clinical studies

Trial ID	# Patients	Aim	Phase	Design	Status
CR-GVH-001	19	Dose finding	Phase I/II	Open-label, dose- escalation	<ul style="list-style-type: none"> Completed 5 year follow-up completed
CR-AIR-006	158	Control group		Observational cohort trial	<ul style="list-style-type: none"> Completed Data analysis
CR-AIR-007	23	Safety and efficacy	Phase II	Open-label, multi-center trial using optimal ATIR101 dose	<ul style="list-style-type: none"> On-going All patients enrolled
CR-AIR-008	15	Dose Optimization	Phase II	Open-label, exploratory trial using repeat dose administration	<ul style="list-style-type: none"> On-going First patient enrolled and treated



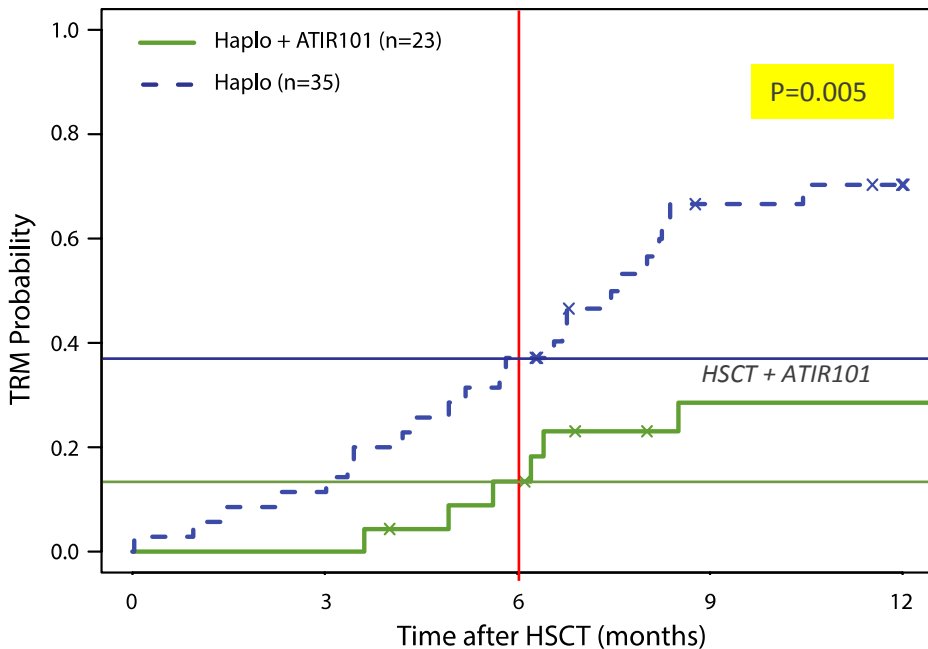
Planned Clinical Studies 2016

CR-AIR-009	Approx. 200	Safety and efficacy	Phase III	Randomized clinical trial: Haplo + ATIR101 (KIADIS) vs. Haplo + Cyclophosphamide (Baltimore)
CR-BD-001	Approx. 10	Safety and efficacy	Phase II	Exploratory Phase II trial in pediatric patients suffering from β -Thalassemia major

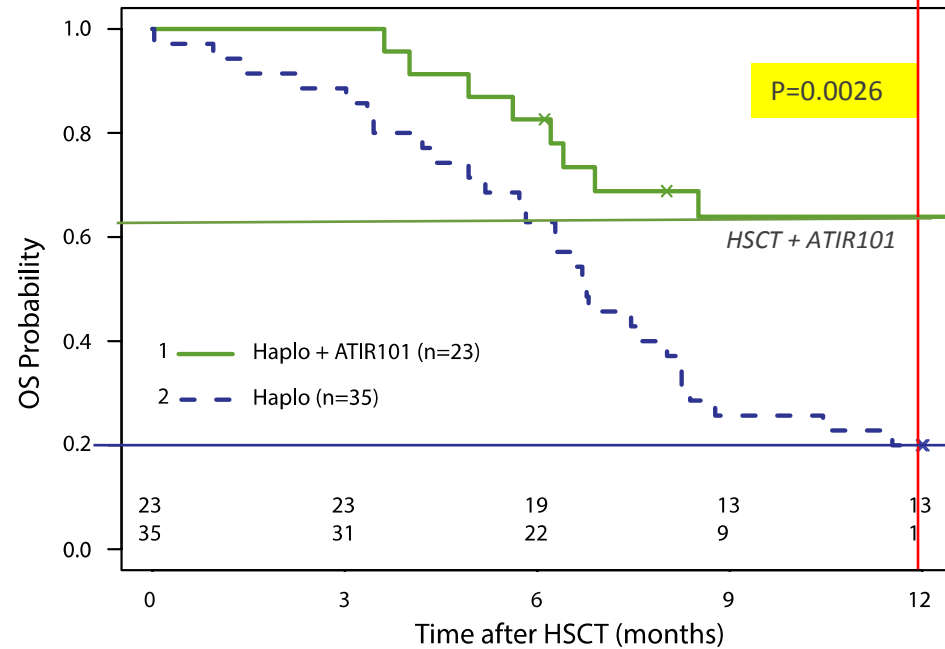
Phase II (CR-AIR-007) data

Control data from an observational cohort study

Transplant Related Mortality (TRM)



Overall Survival (OS)



ATIR101 significantly reduces TRM and improves OS compared to CD34+ haplo-transplants

Regulatory strategy update

EMA

- Process of Marketing Authorization Application (MAA) submission started, Rapporteurs appointed
- Meetings held with Rapporteur and co-Rapporteur
- Decision taken to prepare for MAA filing in 1Q 2017 (awaiting 1 year results CR-AIR-007, dossier writing, completion process validation)
- New ODD submitted: treatment in hematopoietic stem cell transplantation (covers all indications)

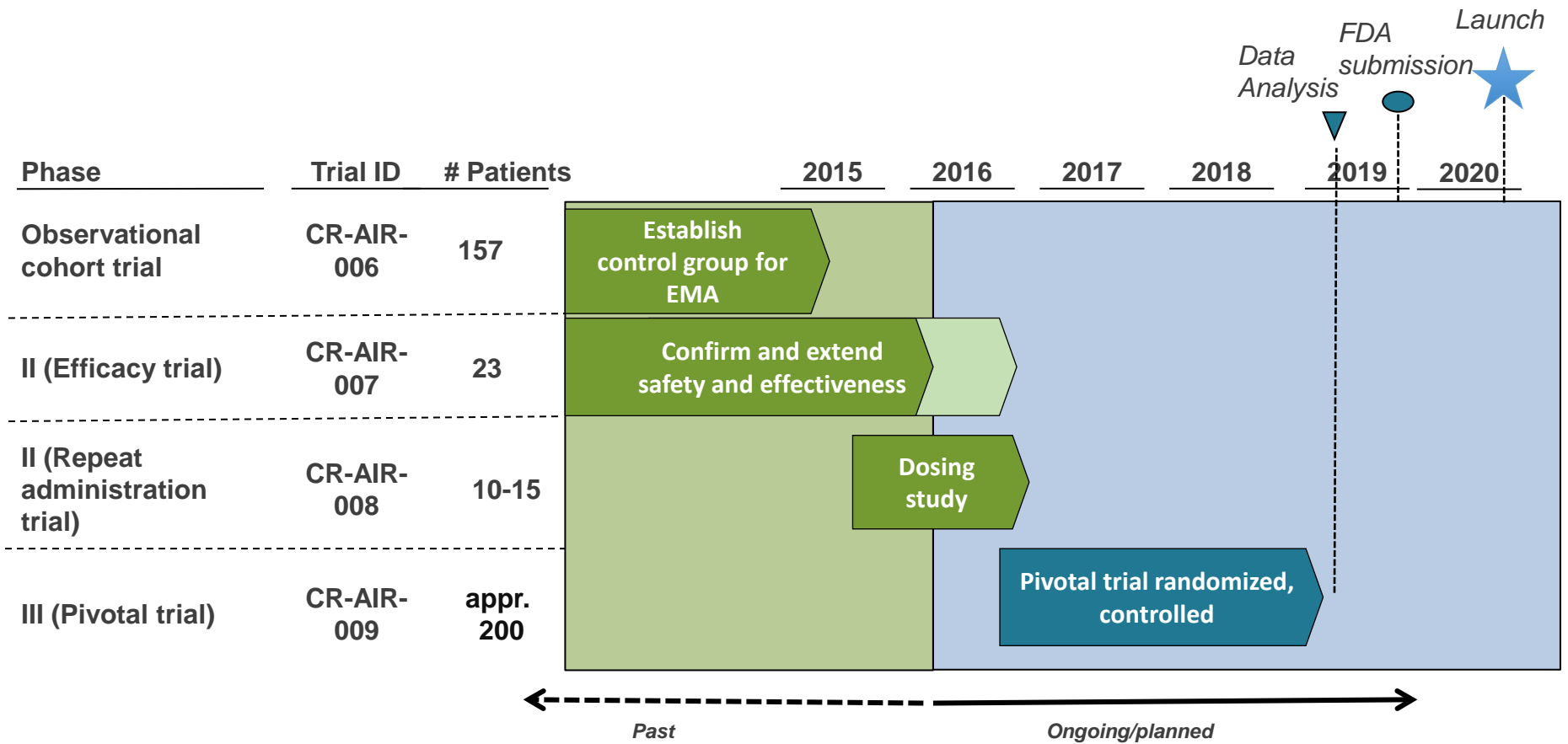
FDA

- End-of-Phase II meeting held in June 2016

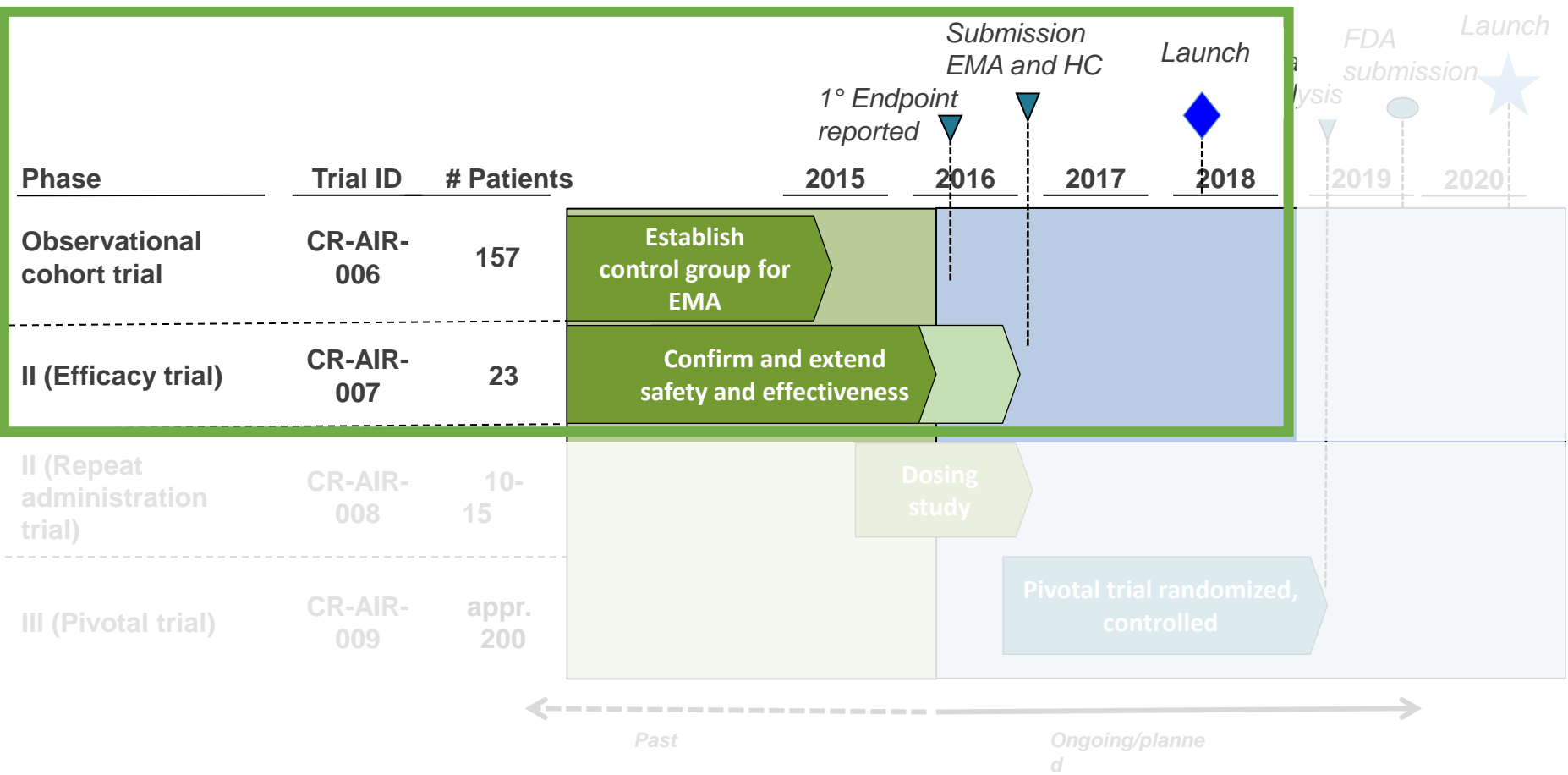
Health Canada

- Possibility to submit MAA to be discussed 2H 2016

Route to market in EU & US for ATIR101

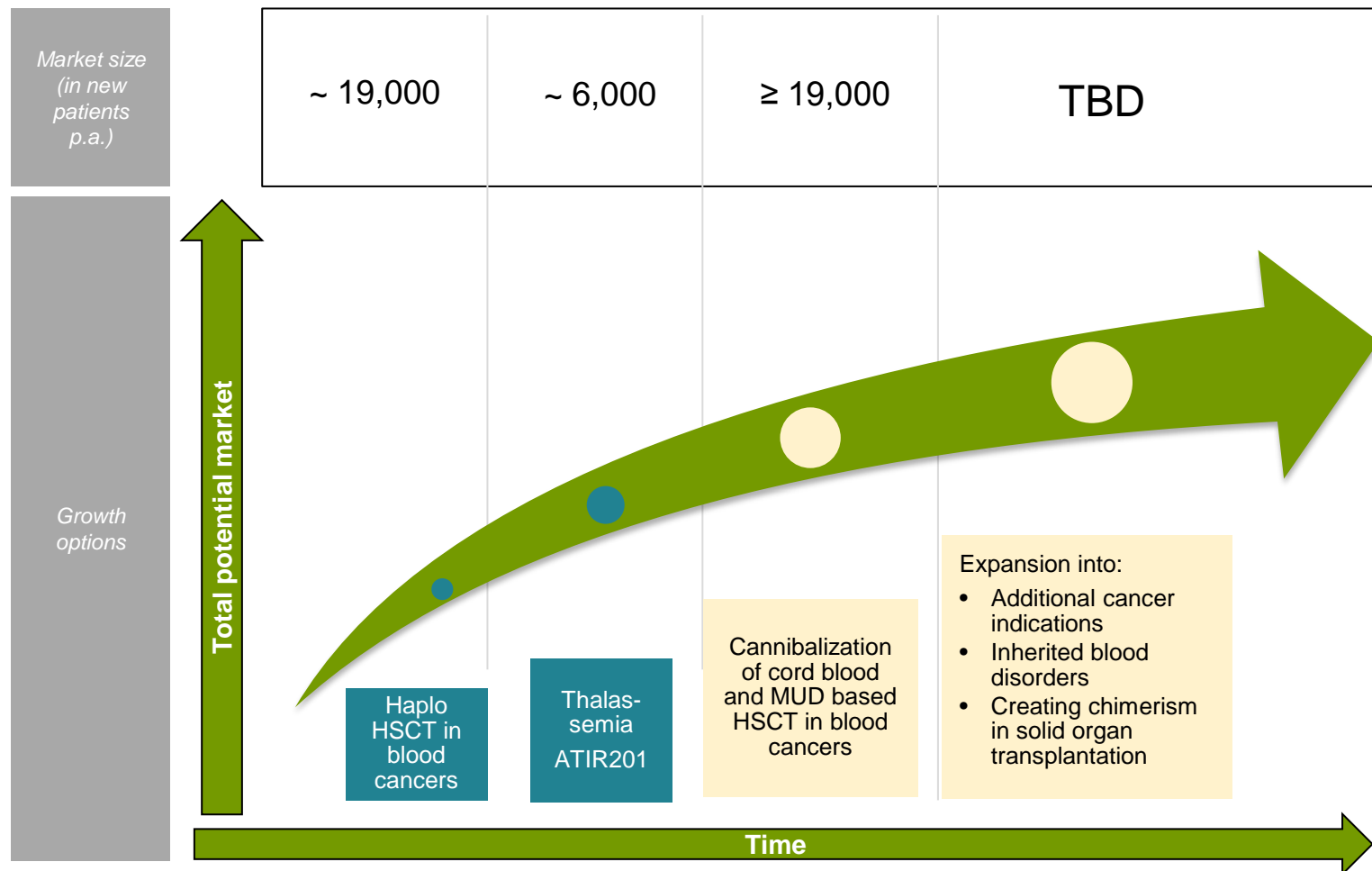


Route to market in EU & US for ATIR101



ATIR may be used in additional indications

Growth options and market size ATIR



ATIR201 in β -Thalassemia major: current therapeutic environment

Current approaches in β -Thalassemia major:

Symptomatic approaches (lifelong):

- Standard of care is symptomatic: blood transfusions and iron chelators
- Drug support to improve hematopoiesis

Curative approaches (by intent):

- Allogeneic bone marrow transplantation to replace the diseased system:
 - Limited availability of matching donors or healthy sibling donors
 - Significant/high risk of infectious complications and GVHD
- Gene-therapy based autologous approaches (e.g. Bluebird Bio):
 - Unknown duration of gene expression
 - Unknown level of gene expression
 - Risk of proto-oncogenic insertion of gene into genome

Clinical program ATIR201

- **Collaboration with TIF**

- Awareness on HSCT option (specifically haploidentical)
- Access to families and to HSCT



- **CR-BD-001**

- Exploratory Phase I/II trial in pediatric patients with β -Thalassemia major
- ATIR201 as adjuvant to a T-cell depleted haploidentical HSCT
- Optimize manufacturing for pediatric setting
- Expected centers: Regensburg (Germany), London, Manchester, Birmingham (UK)
- Protocol drafted
- Trial to be initiated in 2H 2016
- First results expected in 2017, full read-out in 2018

Company (expected) milestones 2016

1H 2016

- Topline results announced CR-AIR-007 trial (ATIR101)
- Set-up US-based ATIR101 manufacturer for Phase III
- US Leukemia and Lymphoma Society takes equity stake in Kiadis

2H 2016

- Opening US clinical sites and strengthening US footprint and presence
- Read-out (safety) of CR-AIR-008 trial
- First patient to be enrolled in ATIR101 Phase III trial (CR-AIR-009)
- Initiation ATIR201 Phase I/II β -Thalassemia trial (CR-BD-001)
- Finalize dossier for submission to EMA for (conditional) approval ATIR101 (Q1, 2017)





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Financial Highlights 2015

AGM, 28 June 2016

Financial highlights 2015

- Shares listed via an initial public offering (IPO) on Euronext Amsterdam and Euronext Brussels on 2 July 2015.
- Raising gross proceeds of EUR 34.7 million and net proceeds of EUR 31.2 million.
- The equity position of the Company improved significantly and increased to EUR 25.7 million at year-end 2015 compared to EUR 2.7 million at the end of 2014.
- Operating loss increased to EUR 16 million in 2015 from a loss of EUR 6.2 million in 2014.
- Operating expenses for 2015 included non-cash share-based payments of EUR 7.8 million.
- Net loss increased to EUR 16.5 million in 2015 from EUR 7.8 million in 2014.
- Net cash used in operating activities and investing activities came to a total of EUR 8.2 million. Net cash from financing activities was EUR 31.2 million positive.
- The Company's cash position came to a level of EUR 28.7 million at year-end 2015, compared to EUR 5.7 million at the end of 2014.



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Thank you