2 SYNOPSIS

This is an addendum synopsis to the synopsis of the abbreviated clinical study report (aCSR).

Study ID:	CR-AIR-009
Phase:	III
Countries:	Belgium, Canada, Croatia, Portugal, Spain, United Kingdom, Netherlands, United States
Study title:	A phase III, multicenter, randomized controlled study to compare safety and efficacy of a haploidentical hematopoietic stem cell transplant (HSCT) and adjunctive treatment with ATIR101, a T-lymphocyte enriched leukocyte preparation depleted *ex vivo* of host alloreactive T-cells, versus a haploidentical HSCT with post-transplant cyclophosphamide (PTCy) in patients with a hematologic malignancy.
Study period:	Study completion date (Last patient last visit): 09 November 2021
Patients analyzed:	All patients randomized in study CR-AIR-009 who underwent HSCT and received treatment with either PTCy or ATIR101 (modified intention-to-treat population [MITT]) and who were still in the study at the end of the active study phase were asked to participate in the long-term follow-up (LTFU) up to 2 years after HSCT. For efficacy, all data of the MITT (not limited to the LTFU period) are included up to 24 months for GRFS, TRM, OS and PFS.
LTFU Patient exposure:	8 treated with ATIR101 and 22 treated with PTCy up to 24 months post HSCT.
Summary of LTFU results:	**Efficacy data for all patients treated with ATIR101 or PTCy including LTFU data up to 24 months post-HSCT:** • On the primary endpoint graft-versus-host disease, relapse-free survival (GRFS), probability was estimated to be 25% for ATIR101 treated patients compared to 62% for PTCy treated patients; • On the secondary endpoint transplant-related mortality (TRM), probability was estimated to be 44% for ATIR101 treated patients compared to 15% for PTCy treated patients; • On the secondary endpoint overall survival (OS), probability was estimated to be 49% for ATIR101 treated patients compared to 77% for PTCy treated patients; • On the secondary endpoint progression-free survival (PFS), probability was estimated to be 44% for ATIR101 treated patients compared to 73% for PTCy treated patients. **Safety:** During the long-term follow-up, of the 8 patients who were treated with ATIR101, one possibly related serious adverse event (SAEs) of grade III/IV acute GVHD was reported which resulted in the death of the patient. From the 22 patients who were treated with PTCy, 1 fatal SAE due to disease relapse was reported which was unlikely related.

Conclusion	The LTFU safety data up to 24 months post HSCT do not reveal any additional safety concerns and are in line with the safety results obtained during the active study phase. Superiority on the primary endpoint GRFS for patients treated with ATIR101 when compared to patients treated with PTCy was not reached at 24 months follow-up for patients participating in the LTFU. This confirms the findings obtained during the active study phase.