

Prognostic factors/outcome of Multiple Myeloma Patients enrolled in European clinical trials after long-term follow-up

Pilot project of the HARMONY Alliance Multiple Myeloma Community

HARMONY: Enabling Better and Faster Treatment for Patients with Blood Cancer.

Facts & Figures

- **Start Date:** 01/01/2017 | **End Date:** 31/12/2021
- **Contributions:** IMI Funding € 20 200 000
- **EFPIA in kind** € 19 094 265 | **Other** € 451 352
- **Total Cost:** € 39 745 617

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Envisioned results

- **R-ISS (Revised-International Staging System)** validation after an extended follow-up and comparison with other prognostic factors.
- **Outcome definition** of patients with low and high-risk features treated with different novel agents and treatment approaches.

Challenge



HARMONY focuses on Hematologic Malignancies, cancers with unmet clinical needs such as Multiple Myeloma (MM). HARMONY has developed a data platform that will register outcome measures that are relevant to different groups of stakeholders, such as physicians, patients, data scientists, drug developers, policy makers and regulators.

A better definition of patients' prognosis has only partially guided therapeutic choices and influenced the outcome of high risk patients that still remains unsatisfactory.

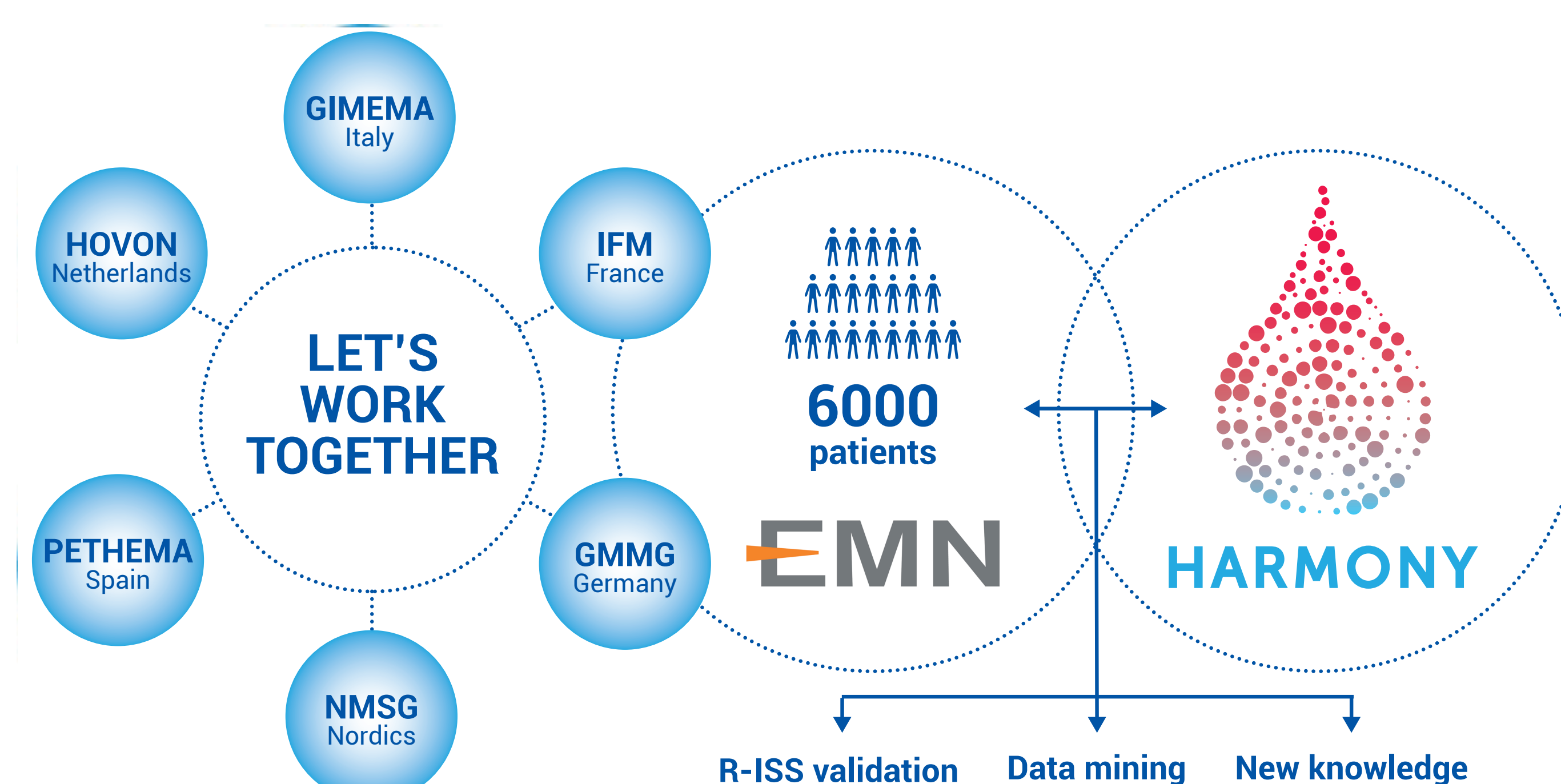
This patients' population represents an unmet clinical need even in the novel agent era.

Approach & Methodology

MM-specific pilot project

European cooperative groups are working together to provide an extended follow-up of the original trials included in the R-ISS project adding other relevant datasets with mature data from clinical trials enrolling NDMM patients treated with novel agents. Endpoints:

- To validate R-ISS, comparing it with ISS, CA and LDH levels alone after an extended follow-up;
- The analysis of the outcome of patients with low and high-risk features (defined according to R-ISS, ISS alone, CA alone, LDH alone, baseline creatinine clearance, best response to therapy) treated with different novel agents (i.e. thalidomide, bortezomib, lenalidomide) and different treatment approaches (i.e. autologous stem cell transplantation (ASCT) vs no ASCT, fixed duration of therapy (FDT) vs continuous therapy (CT)).



Revised-ISS

The clinical outcome of MM patients is heterogeneous. International staging system (ISS), chromosomal abnormalities (CA) detected by interphase fluorescent in situ hybridization (FISH) and serum lactate dehydrogenase (LDH) predict prognosis in newly diagnosed MM (NDMM).

Impact

Prognosis prediction is still an open issue in MM, the outcome of high risk patients is still unsatisfactory and a risk-adapted therapy is not a standard therapeutic approach. The Harmony Alliance provides a unique opportunity to collect data from different European cooperative groups trying to shed light on this important issue and to improve patient management. Supplementing the available data sets with toxicity data, real-life registry data, minimal residual disease data, molecular data and omics data during the course of HARMONY will enable additional analyses and further refinement of MM management.

Our Public-Private Alliance Partners and Associated Members are bringing together their best people, data and knowledge to solve key questions on blood cancers.

Value of IMI collaboration

- Largest European Public-Private Partnership in bigdata & health
- Uniting 53 Partners and 27 Associated Members
- Dedicated Patient cluster
- Collaboration with European Myeloma Network (EMN)
- Data sharing between European Medical Centers and Universities
- Multi stakeholder Community