First Targeted Therapy to FIGHT Hemophagocytic Lymphohistiocytosis (HLH): A novel approach to HLH

From 2012-07-01 to 2017-07-31, closed project

Project details

| Total cost: | EU 11 241 261,60 |
| Topic(s): | HEALTH.2012.2.4.4-1 - Preclinical and/or clinical development of substances with a clear potential as orphan drugs |
| EU contribution: | EUR 5 946 261,80 |
| Funding scheme: | CP-FP - Small or medium-scale focused research project |

Objective

Hemophagocytic lymphohistiocytosis (HLH) is a rare, devastating disease characterized by uncontrolled immune response that primarily affects young infants and children. HLH is usually fatal if untreated. Even when treated, the overall survival rate is still as low as 60-70%. No drug has been formally developed for the treatment of this disease and current treatment methods require the use of drugs which have not formally been developed for this indication which have an unfavourable safety profile. The cytokine interferon-gamma (IFNγ) was shown to play a key pathological role in the disease. It has been demonstrated that neutralization of IFNγ with a monoclonal antibody (mAb) reverts the disease and rescues animals from death in murine models of primary HLH. In patients, evidence points towards a correlation between levels of IFNγ and disease activity. In order to target IFNγ in patients with HLH, Novimmune generated and characterized a fully human mAb, NI-0501, that neutralizes the biological activity of human IFNγ. This consortium proposes an adaptive clinical trial with a pilot and a pivotal phase to provide the necessary data for Market Access Authorization for NI-0501 in HLH. The pilot phase will enroll only patients well-known to have a primary form of HLH and who relapsed after having responded to an initial treatment while the pivotal phase will open the recruitment to newly diagnosed HLH patients. The transition between the pilot and the pivotal phase will be governed by strict pre-defined transition rules and will only occur if a favorable benefit-risk profile has been demonstrated. During the course of the pilot phase the consortium will attempt to generate the necessary information to justify the inclusion in the pivotal phase of a broad range of HLH patient (secondary forms of the disease), for which the pivotal role of IFNγ will have been demonstrated thanks to the research activities of members of the consortium.

Related information

Report Summaries

Final Report Summary - FIGHT-FLH (First Targeted Therapy to FIGHT Hemophagocytic Lymphohistiocytosis (HLH): A novel approach to HLH)
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