

PRESS RELEASE

Approved for a cost of \$ 3.386.735 million* Research Funding Award by the Patient-Centered Outcomes Research Institute

A research team at Foundation I.R.C.C.S. Neurological Institute Carlo Besta has been approved for a cost of \$ 3.386.735 million funding award by the Patient-Centered Outcomes Research Institute (PCORI) to conduct a head-to-head comparison of effectiveness of two approved treatments for multiple sclerosis (MS). This is intended to be the first pragmatic multi-centre randomised controlled trial to directly assess the effectiveness of the new oral agents approved for MS (fingolimod/gilenya versus dimethyl-fumarate/tecfidera) on overall disease experience. It will be a randomized trial taking place in clinical care setting and comparing existing therapies on different outcomes thus exploring their specific effectiveness profile in MS. The study will help to address decisional uncertainty currently facing patients and clinicians, about which is the best treatment for each patient. The present study is planned to be conducted in real world-setting. In this frame the ongoing collaboration with patients and stakeholder is pivotal. Novel patient-centered outcomes will be validated and proposed to be adopted into clinical practice and improve delivery of care. In this sense this project identifies potential end-users of study findings, and incorporate strategies to engage patients in the definition of project's outcomes. Patients, family members and delegate of patients' associations, are conceived of as an essential part of the research team, including also clinicians, experts on trial methodology, health policy makers, nurses, psychologists, basic researchers.

This study is planned to be conducted in the USA, Israel and Europe. Area coordinator centers have been already identified. MS centers with expertise in the use of immunotherapies were selected in Italy, Swiss, Germany, Israel, Spain and USA for participation in this study and coordination of recruitments from additional MS clinical centers of the area. Basic researchers will be involved to study potential biological markers of treatment response and monitor disease progression with novel approach.

Dr. Mantegazza Director of the Neuroimmunology Department and of the MS Center at the Fondazione IRCCS Istituto Neurologico C. Besta expresses his proudness for this very important achievement by Dr. Silvia Rossi, Responsible of the MS Research Activities, for the following reasons: 1. It will be the beginning of a new era of pharmacological trials for the MS community in which the real world is explored appropriately; 2. We will have a crucial matching of the clinical and pre-clinical scientific knowledge for MS patients which will boost

¹ Subject to the negotiation of a formal award contract

the activities of the MS Center; 3. Results from this research will be a step forward towards what is now called "Precision Medicine", the future of medicine and clinical neurology.

"This is going to be the first pragmatic trial in multiple sclerosis. I am very excited to contribute to the clinical research from a new perspective. Here we aim at conducting a comparative clinical study preserving the internal validity due to randomization and generalizability due to a pragmatic design." said the Principal Investigator Silvia Rossi, MD PhD "The availability of comparative data from routine practice will help policy makers to efficiently allocate resources and will drive patients and clinicians in shared and informed health decisions."

The study of this team of Foundation I.R.C.C.S. Neurological Institute Carlo Besta was selected for funding through a PCORI funding announcement specifically focused on comparing the effectiveness of treatment options for MS. While there are a variety of therapies available to people with MS, input from a variety of stakeholders helped PCORI identify this as an important topic for research based on the lack of evidence available to help patients and other stakeholders choose among the available care options.

"This project was selected for PCORI funding for its potential to fill an important gap in our understanding of MS treatment. It will provide useful information to help patient and their caregivers weigh the effectiveness of their care options," said PCORI Executive Director Joe Selby, MD, MPH. "We look forward to following the study's progress and working with Foundation I.R.C.C.S. Neurological Institute Carlo Besta to share the results."

This award has been approved pending completion of a business and programmatic review by PCORI staff and issuance of a formal award contract.

FONDAZIONE I.R.C.C.S. ISTITUTO NEUROLOGICO CARLO BESTA study was selected for PCORI funding through a highly competitive review process in which patients, clinicians, and other stakeholders joined clinical scientists to evaluate the proposals. Applications were assessed for scientific merit, how well they will engage patients and other stakeholders, and their methodological rigor among other criteria.

PCORI is an independent, nonprofit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers, and clinicians with the evidence-based information needed to make better-informed healthcare decisions. For more information about PCORI's funding, visit www.pcori.org.

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