

neceSSiTy



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NEw Clinical Endpoints in primary Sjögren's Syndrome: an Interventional Trial based on stratifying patients

External Newsletter

Issue 1 - March 2020

www.necessity-h2020.eu

Dear NECESSITY friends,

Despite tremendous efforts and recent promising studies, there is still a high unmet need for patients suffering from primary Sjögren's syndrome. To identify new treatment option, we need to develop better tools for assessing treatment efficacy. NECESSITY was formed within the framework of the Innovative Medicines Initiative to join public-private forces to improve the health-related quality of life of patients. NECESSITY aims to identify and validate new clinical outcome assessments, and to discover and validate prognostic biomarkers, for use in future clinical trials of new drugs in development and to be submitted to regulatory authorities for market approval.

We have now reached the end of the first year of our project and work is in full swing. We are happy to report our progress in this annual newsletter.

Finally, we would like to thank all our partners and colleagues for contributing to an excellent start to the project.

*Xavier Mariette, Project Coordinator, INSERM
Peter Gergely, Project Leader, Novartis*

Highlights of the 1st General Assembly

The project first General Assembly was held in Basel, Switzerland at Novartis headquarters. Researchers presented the on-going activities. The discussions focused on the strategy for the development of the



new clinical endpoints and the importance of integrating patient's reported outcome and clinical significance in the new tool.

The group agreed on plans for the upcoming year and how to best integrate input from stakeholders into other work packages' activities.

Report on 2019 activities



The Biomarkers team focuses on identifying and validating tissue and blood biomarkers predictive of major organ manifestations, organ damage and long term outcomes. These biomarkers will be used to stratify patient and to predict therapeutic responses during future clinical trials. The team has defined a strategy for sample analysis and histology and transcriptomics analysis have started on matched samples. Stratification strategies are discussed within the team.

The Clinical Endpoint team focuses on developing and validating new specific outcome measures and new objective tools for use in future clinical trials of primary Sjögren's syndrome. New sensitive endpoints are developed by assessing which items are sensitive to change after therapy using a data-driven statistical approach. Analysis has started on clinical data collected during previous academic and industry-sponsored clinical trials. In parallel, tools for objective measure of symptoms such as dryness and fatigue have been developed and they are currently in test in pilot studies.



The Clinical trial team focuses on the elaboration of a randomized clinical trial based on stratification of patients and using the novel clinical endpoints. The team defined the objectives, design, population of participants, primary and secondary endpoints, exploratory measures and inclusion and exclusion criteria. The treatments used for the trial are standard immunosuppressant drugs available commercially and frequently used for managing autoimmune diseases. The protocol is being assessed by the National Competent Authorities for obtaining regulatory approval.

The Stakeholder team focuses on engaging with key stakeholders to establish mutually agreed clinical endpoints and biomarkers for regulatory approval and market access. The team organized a workshop to obtain input from patients on the unmet needs in primary Sjögren's syndrome, initial feedback from stakeholders on the approach to endpoint development/validation, and recommendations from regulators, HTA, payers and patients on the optimal approach to gain detailed regulatory and payer feedback to the identification and validation of new clinical endpoints. A roadmap for 2020 has been prepared and includes engaging with EMA. Furthermore, a Patient Advisory Group was created to provide recommendations to the consortium.





The Communication team focuses on the dissemination, communication and exploitation of the knowledge created by NECESSITY.

The project website (www.necessity-h2020.eu) was created and includes a page dedicated to patients. Researchers participated to a number of conferences, workshops and seminars. One paper was published (Hammenfors *et al.* *Juvenile Sjögren's Syndrome: Clinical Characteristics With Focus on Salivary Gland Ultrasonography*. **Arthritis Care Res.** 2020) and two manuscripts have been submitted.

Partner in the spotlight: AFGS

Founded in 1990, the **Association Française du Gougerot-Sjögren et des Syndromes Secs (AFGS)** is a French non-profit organization which provides information on primary Sjögren's syndrome and dry syndromes and funds medical research projects. Importantly, it brings together patients and their families and provides a platform for patients to share their daily life experiences and challenges and bring moral support to one another. As a partner to NECESSITY, AFGS guarantees that the project activities address the patients' needs and preferences.

To collect the patient's views, AFGS has created a Patient Advisory Group (PAG)

including patients from most countries participating in the clinical trial. The PAG is contributing to every aspects of the project, from clinical endpoint development to engagement with regulators. They are also participating to the communication activities and have developed a page dedicated to patients on the project website, and are developing a brochure aimed at presenting the project to patients, their family, and the general public. AFGS is represented by **Maggy Pincemin** who is a biology teacher and engineer and has been in charge of AFGS International Relations since 2009.



Learn more about primary Sjögren's syndrome

Primary Sjögren's syndrome is a chronic systemic autoimmune disease causing extreme dryness in mouth and eyes. Patients often experience joint pain and swelling and extreme fatigue, and other organs may be affected. About 5% of patients develop lymphoma, frequently located in salivary glands. Primary Sjögren's syndrome is the second most prevalent rheumatologic disease and mainly affects women over 40 years old. Extreme fatigue and dryness are the main cause for social and professional isolation leading to anxiety and depression.

The cause of the disease is unknown and there is no cure. Only symptomatic treatments are available and they are moderately effective. Consequently there is a high unmet need for new effective treatments.

For more information on the disease, please consult:

www.afgs-syndromes-secs.org

www.sjogrens.org

Upcoming events

EULAR conference

3rd-6th June 2020 - Frankfurt, Germany

The aim of the European Congress of Rheumatology is to provide a forum of the highest standard for scientific (both clinical and basic), educational and social exchange between professionals involved in rheumatology, liaising with patient organisations, in order to achieve progress in the clinical care of people with rheumatic diseases.

www.congress.eular.org/index.cfm



Scandinavian Congress of Rheumatology 2nd-5th September 2020 - Ålesund, Scandinavia

The 38th Scandinavian Congress of Rheumatology, 2nd-5th of September 2020, will take place in Ålesund, on the north-western coast of Norway.

The aim of the meeting is to develop a cutting edge program with broad appeal, and hopefully the SCR2020 will stimulate the intellect as well as the urge for exploration, with inspiring lectures in rheumatology combined with excursions in the city and the surrounding area.

<https://scr2020.no>

List of partners

