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Advances in Knowledge in Niemann-Pick Disease Type C: Facts and Perspectives- 2nd Edition

Guest Editors:

Prof. Dr. Mercè Lliberia

Dr. Daniel Ortuño-Sahagún

Dr. Christian Griñan- Ferre

Deadline for manuscript submissions:
closed (30 November 2021)

Message from the Guest Editors

Niemann–Pick disease Type C (NPC) is an autosomal recessive neurodegenerative disease with a progressive and fatal outcome. Due to its low incidence, i is classified as rare disease, with no effective treatment so far. Today, the denomination designates disorders characterized by unique abnormalities in intracellular cholesterol transport by endocytic trafficking with sequestration of unesterified cholesterol in late endosomes/lysosomes. However, significant advances that led to the elucidation of this disease occurred after the description of the two underlying genes NPC1 and NPC2, with 95% of cases associated to mutations in NPC1.

This Special Issue is focused on the breakthroughs on NPC knowledge from a molecular point of view up to the therapeutic approach. Not only is basic research in animal models necessary to dissect the role of the NPC1 gene in physiological and pathological conditions, but also applied clinical research is mandatory in order to reach the cutting edge of scientific advances that will finally benefit patients, and the sooner this happens, the better.

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Editor-in-Chief

Prof. Dr. Maurizio Battino
Department of Odontostomatologic and Specialized Clinical Sciences, Sez-Biochimica, Faculty of Medicine, Università Politecnica delle Marche, Via Ranieri 65, 60100 Ancona, Italy

Message from the Editor-in-Chief

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International Journal of Molecular Sciences Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland

Tel: +41 61 683 77 34
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