

Journal: Current Neuropharmacology

Title of the proposed Thematic Issue:

Emerging neurobiological concepts and therapies for neurological rare diseases

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From the etymological point of view, the name rare disease incorporates a statistical definition of a disease that affects a small number of the population (1 person per 2000 in Europe). However, from medical point of view, neurological rare diseases include a plethora of complex and heterogeneous conditions, of which the knowledge regarding natural history, diagnosis, prevention, neurobiology and treatment/drug development, is typically limited.

In the last decade, structured data generation and collections, large epidemiological studies, cutting-edge research approaches and technological advances in drug delivery systems, increased our knowledge on the underlying mechanisms of neurological rare diseases, resulting in the development of novel therapeutics.

In the thematic issue under the title: "Emerging neurobiological concepts and therapies for neurological rare diseases", we intend to collect scientific contributions in the field of neurology and neuroscience in search of new neurobiological concepts and novel therapeutic applications in rare diseases of the nervous system. We intend to include hypothesis, scoping and systematic reviews, meta-analyses and mini-reviews for various neurological disorders including sleep disorders, neuropathies, neuromuscular, mitochondrial and neurodegenerative disorders and immune-mediated disorders of the CNS.

Scope: The update on current knowledge and hypotheses on the neurobiology as well as on novel therapeutics in the field of neurological rare diseases.

Sub-topics:

- Novel neurobiological mechanisms and treatments in rare sleep disorders
- molecular-genetic mechanisms and novel therapeutics for rare genetic/inherited diseases of the peripheral nervous system (PNS)

- Treatment advances in immune-mediated neurological disorders
- Replacement therapies & Drug developments targeting pathways in rare neuromuscular disorders
- Advances in the discovery of rare genetic risk factors in neurodegenerative disorders
- Update in rare vascular diseases

Tentative titles of the articles

Sleep Medicine

- Pharmacologic and nonpharmacologic management of central hypersomnia disorders
- Violent behavior during sleep within the context of epilepsy and sleep disorders: Is there a common neurobiological background?

Neuropathies

- Drug and Gene Delivery Systems for treating ATTR neuropathy (transthyretin amyloid neuropathy): Translational Approaches from bench to clinical implementation
- Emerging treatments for CMT neuropathies

Immune system

- Evidence-based cause-cure treatments for immune-mediated cerebellar ataxias

Neuromuscular disorders

- Replacement therapies & Drug developments targeting pathways in rare neuromuscular disorders

Neurodegeneration

- Rare gene variants in Parkinson's disease: a systematic review

Vascular diseases

- *Fabry disease: an update on pathology and management*

General

- Multi-omic approaches for the diagnosis of rare neurological disease

List of potential contributing authors

Special guest contribution:

Claudio Bassetti, Department of Neurology, University of Bern, SWITZERLAND (Scholar h-index: 98)

Georgios Tsivgoulis, National & Kapodistrian University of Athens, GREECE (Scholar h-index: 62)

Marios Hadjivassiliou, Professor of Neurology at Sheffield Teaching Hospitals NHS Foundation Trust, UK (Scholar h-index: 54)

Country contribution

Georgios Hadjigeorgiou, University of Cyprus, CYPRUS (Scholar h-index: 58)

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