**TITLE**

*Lysosomal-Autophagy System Functioning and Diseases*

**Overview of the course**

*Lysosomal storage diseases are a group of metabolic disorders caused by the deficiency of several components of the lysosomes with consequent impairment of the autophagic mechanisms. Although classified as rare, they are devasting disorders often affecting the central nervous system for which no curative treatments are available at the moment. The understanding of the molecular mechanisms of the lysosomal autophagy system functioning and their deregulation in the pathogenesis of lysosomal storage and neurodegenerative diseases pave the way for the development of novel therapeutic strategies for these incurable disorders. The course will cover the main features of the lysosomal autophagy system in physiology and diseases.*

**References**

1. Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome. N Engl J Med. 2021 Nov 18;385(21):1929-1940. doi: 10.1056/NEJMoa2106596.
2. *Early onset effects of single substrate accumulation recapitulate major features of LSD in patient-derived lysosomes*. iScience. 2021 Jun 10;24(7):102707. doi: 10.1016/j.isci.2021.102707.
3. *Heparan sulfate proteoglycans: The sweet side of development turns sour in mucopolysaccharidoses*. Biochim Biophys Acta Mol Basis Dis. 2019 Nov 1;1865(11):165539. doi: 10.1016/j.bbadis.2019.165539.
4. *Autophagy and Neurodegeneration: Pathogenic Mechanisms and Therapeutic Opportunities.* Neuron. 2017 Mar 8;93(5):1015-1034. doi: 10.1016/j.neuron.2017.01.022.

**Schedule (~6 hours)**

The course will include three lessons (2 hours each), as follows.

**Program**

**Lesson one (*Prof. Luigi Michele Pavone*): 7, November 2022, 14:30.**

Seminar room 4th floor Torre Biologica

Title: *Lysosomal storage diseases: pathogenetic mechanisms, diagnosis, and novel therapeutic approaches*

**Lesson two (*Prof. Massimo D’Agostino*): 8, November 2022, 14:30.**

Seminar room 4th floor Torre Biologica

Title: *Lysosome functioning and dysfunction in physiology and diseases*

**Lesson three (*Prof. Maurizio Renna*): 9, November 2022, 15:00.**

Seminar room 4th floor Torre Biologica

Title: *Regulation of (macro)autophagy: the importance of being a (possibly) healthy lysosome in neurodegenerative and infectious diseases*