

# **Gordon Research Conferences**

## *frontiers of science*

### **Lysosomal Diseases**

#### ***Understanding Lysosomal Biology and Disease Mechanisms to Develop New Therapies for Lysosomal Diseases***

In the early '50s, Christian De Duve identified a new cellular structure, the lysosome, defined as the cell's "suicide bag". Sixty years later, it is clear that the lysosome greatly exceeded the expectations of its discoverer. Recent findings on the role of the lysosome in endocytosis, exocytosis, autophagy, nutrient sensing, signaling and on the mechanisms underlying lysosome positioning changed our traditional view of the lysosome from a dead-end organelle to a control center of cell homeostasis. Over 50 different types of lysosomal storage diseases have been identified, each due to the deficiency or malfunction of a specific lysosomal protein. These disorders affect many organ systems, most notably brain, leading to chronic illness and death of affected individuals. Although these diseases were among the first for which both the biochemical and the molecular basis were recognized, the mechanisms by which the accumulation of storage material in lysosomes translates into cellular and tissue dysfunction and clinical symptoms has yet to be fully elucidated. In addition, an important role of the lysosome has been unveiled in several common human diseases, such as cancer, obesity, neurodegenerative diseases, and infection, identifying a "new class" of lysosomal diseases. An important challenge for the future will be to exploit recent discoveries to better understand the mechanisms underlying lysosomal diseases and to develop new treatments. The Lysosomal Disease GRC aims to stimulate interactions among experts in the field to improve knowledge about the pathophysiology of lysosomal diseases and identify innovative diagnostic and treatment approaches. This fourth Gordon Research Conference on Lysosomal Diseases will be held at the Renaissance Tuscany Il Ciocco Resort in Lucca (Barga), Italy, which will offer a wonderful venue to address major topics in lysosomal biology, disease mechanisms, diagnosis and therapy.



**March 5-10, 2017**  
**Il Ciocco Hotel & Resort**  
**Lucca (Barga), Italy**

**Chair:** Andrea Ballabio  
**Vice Chair:** Beverly Davidson

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