

***Caenorhabditis elegans* as a screening tool to find active compounds and targets in rare neuromuscular diseases.**

Maité Carre-Pierrat, Jean Giacomotto, Jessica Cisek and Laurent Ségalat

CNRS UMR5534 – Centre de Génétique Moléculaire et Cellulaire

16 rue Raphaël Dubois – bâtiment Mendel

Campus de La Doua – 69100 Villeurbanne – FRANCE

Current high-throughput screening methods for drug discovery rely upon the existence of targets and therefore on the understanding of the pathogenesis of a disease. In the case of diseases where the pathogenesis is not yet known, drug discovery is only feasible on model organisms mimicking the disease. The nematode *Caenorhabditis elegans*, which combines genetic amenability, low cost, and easy culture conditions, is compatible with large-scale screens.

Here, we report the results of screens of chemical libraries on *Caenorhabditis elegans* models of the Duchenne Muscular Dystrophy, the Spinal Muscular Amyotrophy and the Schwartz-Jampel syndrome. We present the methodology used for each model to screen up to 7,000 compounds and the results of these screening campaigns. We further present the validation of our best hits in mammalian models and try to understand their mechanism of action.