

## The disease modelling value of baby teeth: A new way to unlock knowledge about a special group of genetic disorders

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Mucopolysaccharidoses (MPS), are a group of genetic, metabolic, and rare diseases investigated since the early years of the 20th century. One of the first steps to collect information about the underlying mechanisms of those disorders is the development and analysis of in vitro models. Furthermore, those models provide an appropriate platform for the evaluation of future therapeutics. Among all the possible disease cell models, patient-derived ones are those which allow us to get better disease insights. However, finding the best cell type that recapitulates disease-relevant features is not always easy: two systems largely involved in MPS pathology are the brain and the musculoskeletal ones, which reflects an issue once both are hard to access.

Here, our main goal is to establish an innovative non-invasive method to generate disease-relevant cell models from stem cells from deciduous (baby) teeth (SHED), which may then be differentiated into our MPS-target cell lines.

So far, we have already implemented and optimized the protocol for collection, isolation, establishment and cryopreservation of those stem cells. Then, our rationale is simple: for those obtained from MPS patients suffering from multisystemic disease with marked musculoskeletal alterations, we are using a chondrogenesis differentiation protocol. For those derived from patients with neurological pathology, we will establish mixed neuronal/glial cultures. As soon as we can get the SHED-derived differentiated cells, various cellular and molecular processes from our target disorders may be unveiled and used as a target for possible future therapeutics.

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