

## **A Biobank of APBD patient skin fibroblasts**

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We have generated a novel, personalized analytical tool for assessing drug efficacy. This tool is image-based phenotyping (IBP): a global phenotypic fingerprint of patients' primary skin fibroblasts, which encompasses multiple cellular and subcellular features. IBP enables us to identify image based features unique to APBD patient cells, which may change in response to tested small molecules, or any other drug for that matter. This analysis has generated phenotypic signatures of APBD patients and control subjects. The accuracy of these signatures improves as skin fibroblasts from more patients are analyzed. Using computational classification methods, we are able to define control and pathological global phenotypes in skin fibroblasts respectively derived from unaffected subjects and APBD patients. These IBP signatures will enable us to **a)** test any therapeutic candidate for its ability to turn a global APBD phenotype into a global unaffected phenotype; and **b)** predict adverse or side effects of any therapeutic candidate based on its effect on multiple cellular features.