

Diamond-Blackfan Anemia is a genetic disorder characterized by a failure to produce red blood cells. While there are a few options for patients, the currently available treatments carry substantial risk and side effects. We have established stem cell-based models that replicate the maturation of red blood cells within the human body, allowing us to gather clues about the biological mechanisms at play. Based on our results so far, we propose a unique biological explanation for Diamond-Blackfan anemia. Our focus now includes both gene therapy-based approaches (genetic manipulation of the patient's DNA in order to repair the mutation that causes DBA) and the generation of a new class of protein-based drugs. We are currently working to validate these new therapies, with hopes that, if they continue to show promise, we will be able to move forward towards human clinical trials in the next few years.