

Amendment history for PP1533650

July 2022

- Addition of Schwachman Diamond Syndrome as a target disease area with an addition of a protocol (8)

September 2022

- Addition of an optional step to dose animals with a tracer compound on protocol 8

January 2023

- Change of PPL holder due to the previous holder leaving RxCelerate

April 2023

- Addition of hydrodynamic administration of gene expressing vectors to protocol 6
- Increase in weight loss limits from 10% to 15% and therefore severity from mild to moderate on protocol 8.

September 2023

- Amendment of adverse effects and humane endpoint related to hydrodynamic injection following a change in recommendation to carry this procedure out under anaesthesia which led to altered adverse effects and a slower recovery time from the procedure

October 2023

- Increase to the weight loss limit on protocol 8 following studies showing that to create an appropriate therapeutic window weight loss of up to 20% without other clinical signs may be required.

August 2024

- Clarification of weight loss humane end points across all protocols
- Addition of protocols related to fibrosis (Protocol 9: lung, protocol 10 skin, protocol 11: kidney) and rheumatoid arthritis (protocol 12)