

From: Pauline Kung [REDACTED]
To: panel_hs@legco.gov.hk

Date: Sunday, April 02, 2017 11:19PM
Subject: Re: policy on and drugs for rare diseases- urge to subsidize the drug for rare diseases

To whom it may concern,

We are the parents of a rare disease patient. Our son, currently 2.5 years old, is suffering from aHUS, a life threatening blood disease that can't be cure, my son has had 2 episodes since last year and had gone through very tough treatment in PICU, which includes plasma exchange and kidney dialysis. It has caused him kidney damages that needs to be closely monitored.

Doctor at Queen Mary hospital has suggested the usage of this medicine, Soliris eculizumab, to replace plasma exchange, because it reduces the risk during treatment, and has been used overseas to help effectively control the relapses of aHUS , the orphan drug exactly the same that CCF is planning to subsidize for PNH patient.

However, the current cost is way too high and we hope that it will be subsidized in the near future so we don't have to face the life threatening situation again, and our son can continue to grow up in a stable health condition.

Thank you for your attention.

Regards,

Pauline Kung and Keng Fok