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POST-MARKET REVIEW OF THE LIFE SAVING DRUGS PROGRAMME

Case:

In June 2014, five batches of Eculizumab were recalled by the FDA (USA) and urgently recalled by TGA (Australia), because the manufacturer indicated there was a potential risk for a stability failure prior to end of shelf life. One of my patients, who has an extreme case of PNH with clone size 99%, received four consecutive doses from one of the recalled batches and relapsed several days before the recall notice was issued. The patient is dependent upon regular access to Eculizumab and relapsed with fulminant haemolysis and no evidence of a clinical precipitant, no leukocytosis and no elevation of CRP level. A single dose of Eculizumab from a non-recalled batch reversed haemolysis and rescued the patient. The rescue dose cost \$18,000.00, paid from the South Australian health budget.

My issues with LSDP are:

1. The LSDP refused to release the clearly needed rescue dose.
2. It was not apparent to me that LSDP understood implications of PNH clone size and its relationship to relapse risk.
3. It was not apparent to me that LSDP consulted any clinicians, who are independent of the supplier, for advice regarding PNH relapse and rescue.
4. It was not apparent to me that LSDP made any effort to recover cost of public used to purchase the recalled batches of drug.
5. It was not apparent to me that LSDP made any effort to recover public money subsequently paid by the South Australian government to purchase the extra rescue dose.
6. It did seem to me that LSDP accepted the manufacturer's statements regarding stability of recalled drug batches without independent scientific or medical review.

In summary, my concern with LSDP is that it does not appear to take initiative when the circumstances vary from the standard process. I expect that many Australian medical specialists with experience treating this rare disease would have been happy to offer advice to LSDP. It appeared to me that LSDP does not engage with specialist clinicians and expert (PhD) scientists to adequately appraise clinical and scientific data that require LSDP review. It appeared to me that LSDP failed to appreciate the significance of a FDA recall and a TGA urgent recall. It appeared to me that LSDP failed to act independent of the manufacturer. It appeared to me that in this instance, LSDP failed to spend Australian tax dollars responsibly.

I am happy to discuss further.