

Submission from Miss NG Hei-tung

I am writing to express my point of view regarding rare disease treatment.

PNH patients have been fighting their advocacy battle for almost 3 years. They are sick, with young kids, old parents to take care of. They have been fighting a hard battle against illness and yet the government keeps procrastinating their access to the drug that have been proven effective in treating them.

There are patients passed away.

There is patient's spouse suffering from emotional stress in taking care of kids.

There is patient's 70 years old parent thinking to sell her only flat for drug to her only son.

There is patient forced to stop working because of volatile health status.

The delay of drug to PNH patients has created enough traumas for patients. That's enough.

PNH patients cannot afford the delay. We are grateful of having PNH as pilot case, yet we hope the government would adhere to the following principles when making the plan ahead:

- **High transparency of subsidy mechanism:** including clinical criteria of using drug, sustainability of subsidy
- **Patient Consultation:** Patient shall be involved in official consultations with different stakeholders including CCF to ensure the mechanism proposed can genuinely address patients' concerns.
- **Patient quality of life:** PNH can lead to various complications. When setting the clinical criteria indicator, patients' quality of life shall be take into consideration. Remember most of PNH patients are breadwinners, their quality of life must be ensured to be able to work.
- **Special assessment criteria for financial mean test on rare disease drug:** Given the rare disease drug is expensive, government shall come up with a reasonable assessment criteria. Remember the PNH patients live with the disease lifelong and the scheme shall take into account of that financial implications for patient & their families.
- **New healthcare funding model:** In our previous correspondence with government, the usual response is "the drug is very expensive and they will conduct assessment based on cost-effectiveness, fair use of resources" etc. We want to reinforce the point that this is government responsibility to take a lead with other key stakeholders to creatively re-engineer our healthcare system that is appropriate with the accelerating medical innovation. Be fair to patients and have them access to drugs in need.