

Patient group consultation main points

Represented by patients from:

- Malaysian Rare Disease Association
- Malaysian Lysosomal Association
- WeCareJourney
- MyPOPI
- Motor Neuron
- Informal Patient groups : parents of children with Marfan Syndrome, Cornelia De Lange Syndrome & Lysosomal Storage Disease

Topic	Points of discussion
Lack of awareness	<ul style="list-style-type: none">● Lack of knowledge has caused patients/families to deal with illness without any support group for a long time● Important to raise awareness for communities that have limited access to knowledge● There is a need to recognise the change in needs when a child patient enter adulthood● Grab drivers should have awareness on people with disabilities● Grab should provide some discount● Print and electronic media are more interested in writing from sensational angle rather than from educational angle
Definition	<ul style="list-style-type: none">● Clarity needed on the disease portion of the definition of rare disease. Need to distinguish between disease and disorder● The definition should be relevant to your country's situation in terms of prevalence and incidence of rare diseases and should be guided by cost saving.● Will Primary Immunodeficiency be included in the definition?
Registry	<ul style="list-style-type: none">● Concern that registry is too loosely defined - In the US, they have a very sophisticated registry containing the diagnosis and everything. In Asia, what we think of as a registry is just the name and number, which may be the necessary next step at this point.● Need a single driver to take the initiative of setting up the registry
Education	<ul style="list-style-type: none">● Children get discriminated at school. Schools need to be educated on these conditions.● It is important that the children/peers are educated too.● Public schools can be stressful for children with Marfan syndrome but special schools are often unaffordable.
Ministry	<ul style="list-style-type: none">● Indifference in MOH on the basis there is no cure and treatment for rare diseases and the high cost of drugs, thus resulting in:<ul style="list-style-type: none">○ no management policy for RD○ no educational pamphlets of RD available to provide the information to educate patients and caregivers and all the service providers both horizontally and vertically.

	<ul style="list-style-type: none"> ○ lack of financial support to help activities of patient groups/organisations to sustain awareness/education programmes for patients/family members/ caregiver and the public ○ No increase in the number of geneticists and creation of genetic counsellors posts to better serve RD patients throughout the country especially adult patients. ○ lack of financial support from corporate sectors to carry out programmes because it does not meet CSR guidelines ● Ministry has banned medication to slow down blood pressure and no substitute medicine has been provided ● UM should be associated with MRDS to form one central and formal centre for the university. ● Malaysia does not have a specific desk officer for rare disease ● Politics within the ministry of health block progress on rare disease ● Immunology not recognised as a subspecialty leading to little incentive to study it
Funding	<ul style="list-style-type: none"> ● Limited financing for medication and treatment ● Aid received under OKU only RM250 and insufficient ● Funding important for MND as they need special devices to breathe. ● Difficulty getting insurance as insurance does not cover diseases that are hereditary. ● Lack of financial support from corporate sectors to carry out programmes because it does not meet their CSR guideline. ● Consider tax exemption for medical devices
Clinicians	<ul style="list-style-type: none"> ● In certain cases, the doctors are not aware of the disease as it is a rare condition and they could not diagnose nor provide care for these patients. ● Doctors do not follow up on diagnosis by recommending the next steps to treat the condition ● Small number of clinicians available in the field ● Need to overcome medical professional's reservation to want to help a small number of people
Take away points	<ul style="list-style-type: none"> ● Important to advocate for low hanging fruit and have a loud voice to gain publicity ● We may need to convince medical doctors as well as public ● Next time we should have a mixed group discussion inclusive of government ● Ensure everything is inclusive. Undiagnosed RD patients also have to be brought in to benefit the report ● Suggestion: to begin a basic patient registry by the patient groups and owned by the patient groups ● Scenario analysis of economic impacts may be necessary to advocate for the adoption of RD policies

	<ul style="list-style-type: none">• We should create a timeline of our efforts as well as what we are doing and where we are doing it in order to coordinate the efforts
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