PHILIPPINE SOCIETY FOR ORPHAN DISORDERS, INC.

Understanding HTA as a Tool for Participation in Policy Decision for Rare Disease Persons (Philippines)

Presented at the
2nd Rare Disease Asia Conference 2016
in Kuala Lampur, Malaysia

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TOPIC COVERAGE

THE CHALLENGE TO UNDERSTAND HTA:
THE PHILHEALTH PROPOSED PRIORITY SETTING INITIATIVE

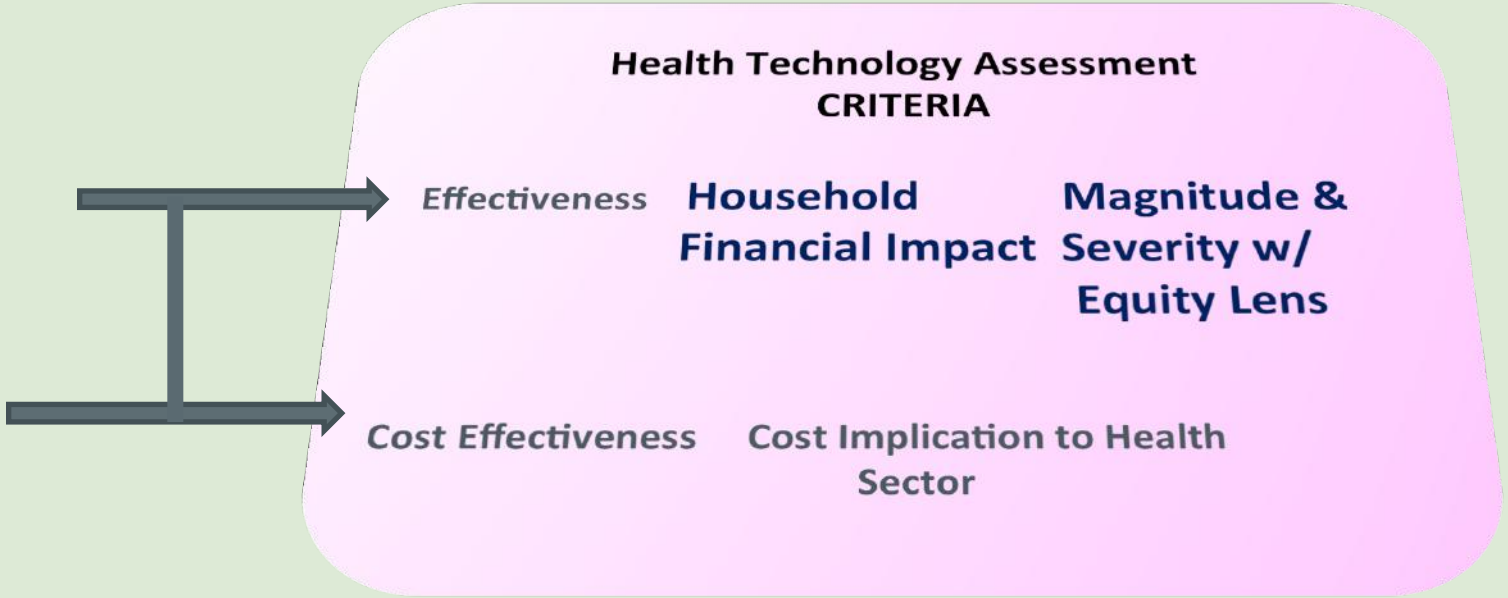
DEVELOPMENT OF PATIENT PROFILE FOR MPS (EVIDENCE BASED SURVEY)
SAMPLE SURVEY OF FINDINGS:
COST OF TREATMENT AND CARE vs. HOUSEHOLD INCOME
CHALLENGES TO QUALITY OF LIFE TO PATIENT AND FAMILIES

TRANSFORMING LIVES (VIDEO PRESENTATION)

REASONS TO HOPE

PHILHEALTH PROPOSED PRIORITY SETTING INITIATIVE PROCESS

1. Disease-Intervention Nomination screening
2. Short Listing of Intervention for Assessment
3. Evidence Generation
4. Evidence Appraisal
5. Approval of coverage
6. Approval of Implementation



BENEFIT PACKAGE DEVELOPMENT

INITIAL STEPS IN GATHERING EVIDENCE BASED DATA

1. HARNESSED THE EXPERIENCES OF PATIENTS AND THEIR FAMILIES AND DOCUMENTING PATIENT STORIES GATHERED OVER THE 10 YEARS . USED THE INFORMATION AS BASIS FOR PREPARING THE SURVEY

2. BENEFIT OF CONSULTING EXPERTS FROM ABROAD WHO HAVE HAD EXTENSIVE EXPERIENCE IN HTA IMPLEMENTATION IN OTHER COUNTRIES

3. PREPARED AND PRETESTED THE SURVEY WITH THE MPS GROUP, MPS, ONE OF THE BIGGER RARE DISEASE GROUP IN OUR REGISTRY IN A GROUP SESSION; REVIEWED THE RESPONSES; CONDUCTED INTERVIEW FOR VALIDATION OF THEIR UNDERSTANDING OF THE SURVEY FOR ACCURACY

4. INITIAL COLLATION OF OF DATA

PRESENTATION OF SAMPLE DATA

A. IMPACT OF THE DISEASE ON HOUSEHOLD FINANCES: HOUSEHOLD INCOME vs. Cost of Treatment and SUPPORTIVE CARE

B. IMPACT OF THE MPS DISEASE ON THE PATIENT'S QUALITY OF LIFE :

- MOBILITY
- MOTOR SKILLS (FINE AND GROSS)
- HEARING
- VISION
- SOCIAL PARTICIPATION/INTEGRATION
- EDUCATION

MPS PROFILE

A. MANIFESTATION OF SYMPTOMS

- Symptoms started between the age of 1 year old to 4 years old and progresses beginning at the age of 7

B. CONFIRMATON OF DIAGNOSIS

- Average period from the time of the manifestation of symptoms to confirmation of condition is 3 to 4 years.
- Average period to confirm the suspicion of an MPS condition took 2 months from the time they were seen by a specialist who suspected that the condition could be rare disorder, and release of diagnostic test results

C. COMMON SYMPTOMS MANIFIESTED AT AGE 1 TO 4 YEARS OLD

- Joint stiffness
- Persistent pulmonary problems
- Noisy breathing
- Hirutism (short structure compared to normal standard)

Summary of Results; Impact of MPS on Quality of Life As of August 2016)

From 21 respondents out of 30 in PSOD Registry

Mobility

6 Children ages 13 to 18 need full assistance to move around with the use of assistive devices

3 Children ages 9 to 11 are still able to walk but need aid or support (without assisted devices)

12 Children have no problem to move around on their own

Motor skills

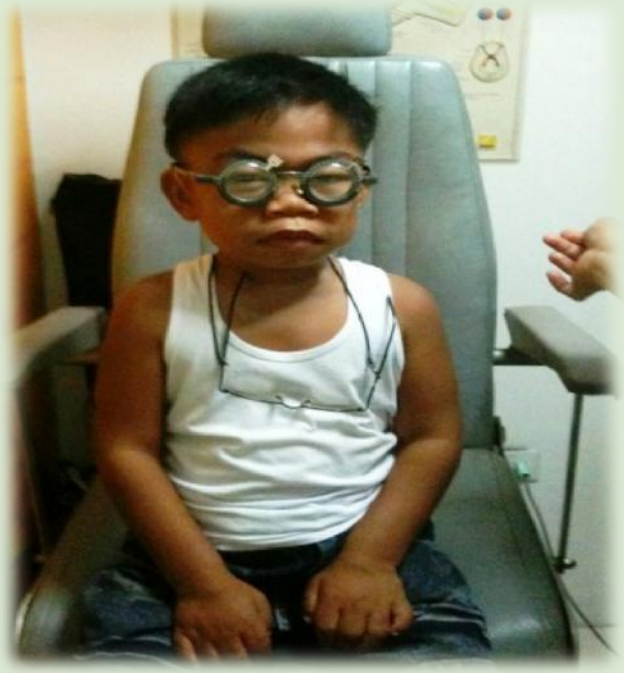
12 children ages 4 to 20 have partially loss their fine motor skills in which they have problems with their grip

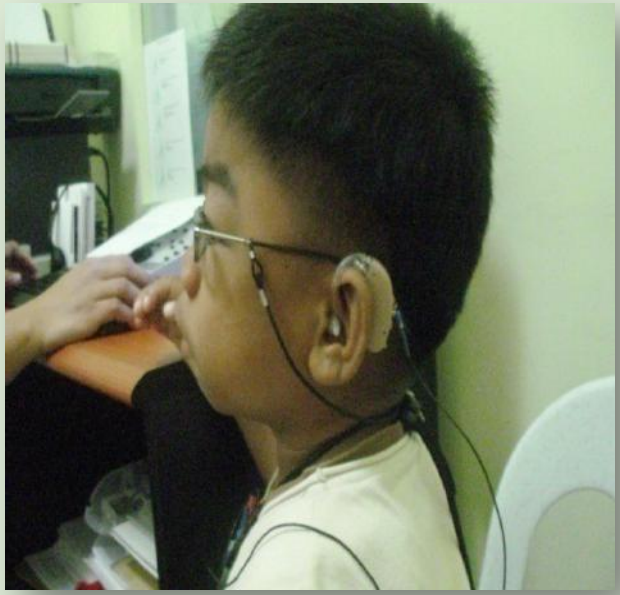
9 children ages 4 to 20 partially has lost their gross motor skills, which varies from extending the arms, standing, sitting or rolling over without aid or help

Hearing

8 Children ages 6 to 20 use hearing aids

3 respondents has severe or sloping to profound hearing loss





VISION

8 children ages 10 to 27 are using progressive eyeglasses

3 Children ages 6 to 16 severe vision problem

COMMUNICATE VERBALLY

5 Children ages 5 to 16 has complete loss to communicate verbally

3 children ages 6 to 10 has difficulties to communicate verbally

SELF CARE/MANAGEMENT

11 children ages 4 to 20 yo have difficulties to undertake self care and manage self. Difficulties varies from feeding self, putting on clothes and or personal hygiene

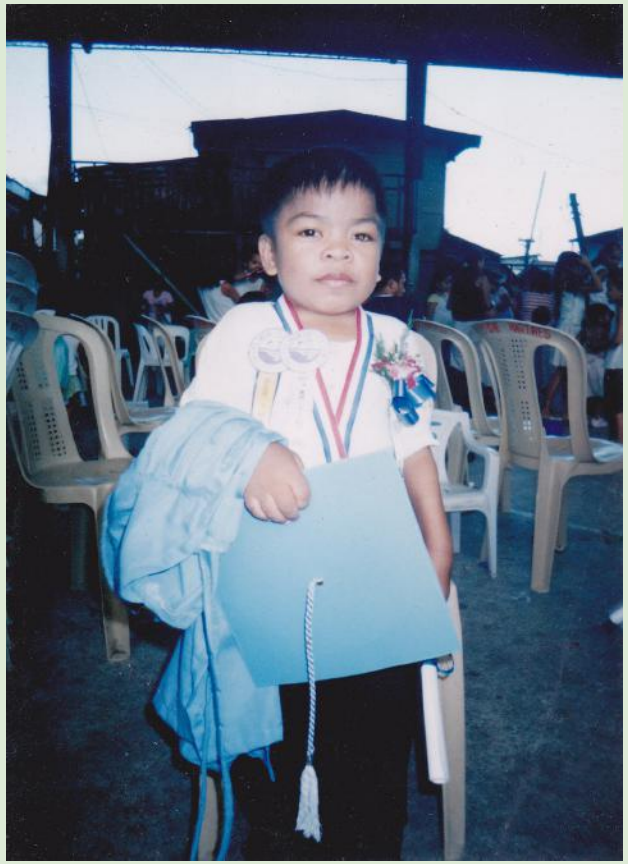
7 children ages 5 to 18 requires full assistance in all areas of care.

SOCIAL PARTICIPATION

4 Children ages 10 TO 18 are unable or have completely lost their ability to integrate in the community because of total dependency

6 children ages 5 to 11 find it difficult to participate, interact and integrate with the community for varied reasons cited , such as low self esteem, experienced bullying and discrimination and loss of all abilities.

7 children ages 10 to 20 are able to actively participate in the community, however, still experienced bullying because of the apparent physical difference of the child.



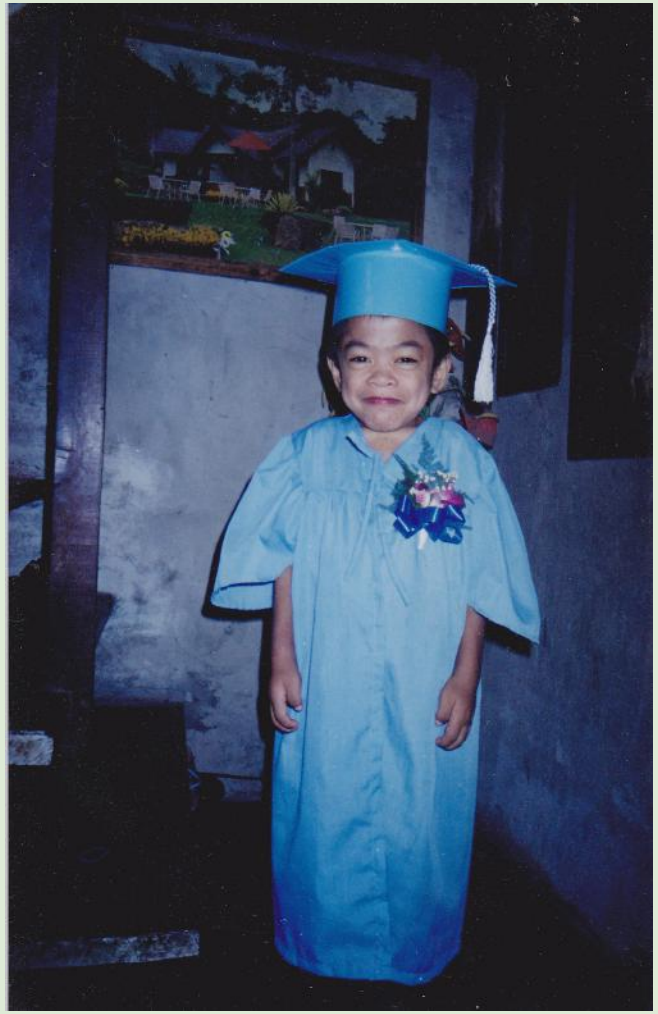
EDUCATIONAL ATTAINMENT

- 7 children ages 11 to 20 have had a certain degree of education but are unable to continue higher studies , for various factors, such as medical, physical challenges and financial challenges; 2 among these individuals were able to finished high school while others unable to continue in grade school.

- 9 children, ages 6 to 27 are currently able to continue their education; 2 are now in college, in spite of the difficulties and barriers even without benefit of treatment

- 4 was not able to attend school at all because of the early progression of the disease

- 1 has not reach school age



Impact of the Disease to the Patient

Without treatment, their condition will progress and other complications arises such as problems in mobility, motor skills, hearing, vision, communicate verbally, self-care, and social participation, continued education

HOUSEHOLD FINANCIAL IMPACT TO ACCESS TREATMENT AND SUPPORTIVE CARE

21 respondents out of 30 total population registered

- Estimated gross family income of 10 respondents: fall between Ph11,000 ($ 224) to Ph20,000/month ($ 407)

- 6 are below Ph11,000 and 5 above Ph 20,000

THE COST OF DIAGNOSTIC TESTS AND MONITORING TEST ALONE WILL CONSUME ALL OR MOST OF THE COST OF THE FAMILY INCOME.

AVAILABLE TREATMENT , IF IT WILL COME FROM THEIR OUT OF POCKET WITH UNSTAINABLE HELP FROM DONORS IS NOT POSSIBLE.

IMPACT on the PATIENT and Families

Transforming Lives
Video Presentation
(2 case Presentation)

CONCLUSION

HTA IS A VITAL TOOL IN PRESENTING EVIDENCED BASED JUSTIFICATION FOR POLICY DECISIONS FOR SUSTAINABLE BENEFIT COVERAGE FOR RARE DISEASE PATIENTS

PATIENT ORGANIZATIONS ROLE IS VITAL IN GATHERING EVIDENCES FOR HTA

Reasons to Hope

1. Early Detection/Early Intervention: Increasing medical awareness thru the efforts of IHG, participation of more specialist who have encountered RD patients ; continuing awareness campaign of families, the patient organization, advocates, media

   Inclusion of more rare disease in the Expanded New Born Screening

2. Inclusion of RD in the Universal Health Program of the Government

4. Passage of RA 1077: The Rare Disease Act of the Philippines (March 3, 2016)

5. Patient Centered Policy of DOH pronouncement where patient voice are represented by patient organization giving way to in policy decisions.

6. Increased interest in other bio pharma in making available their treatment for rare disease in the Philippines

These Children too have an Equal Right to Life
They deserve a Chance in Life
Together We Can Help Them








Care for Persons with a Rare Disorder thru Partnerships

Maraming Salamat Po