Thank you very much for your kind cooperation. We believe our endeavor will lead to the development of a newtreatment.

Dr.Shunji Tomatsu,

The Carol Ann Foundation Registry

Before completing the questionnaire thoroughly read the **consent agreement** and acknowledge your consent. Check the appropriate box indicating if you wish to fill out this form online or offline. This questionnaire is 14 pages long and will take time to complete thoroughly. Please don't rush through it. If you don't know an answer to a question please state so. All answers are important and very vital to Morquio, (MPS - IVA) research. Aims for the Morquio, (MPS - IVA) registry is as follows:

1. Establishment of International Registry and network among Morquio families
2. A summary of the natural history of Morquio disease
3. Education of Morquio families, medical staff and to further research on Morquio disease
4. Definition of clinical endpoint at the clinical trial

The Carol Ann Foundation has full ownership of this registry.

The Carol Ann Foundation (CAF) has asked Dr. Tomatsu, Professor and Director, Skeletal Dysplasia Lab Pediatric Orthopedic Surgery , to summarize and review the data statistically. The summary of this data will be returned to the registerer in the newsletter or in our Monograph on Morquio. We do not give any statistical data to third party or non-registrar except for academic purposes (medical meeting, publication, etc.) permitted by the Board of Directors of CAF.

Although there is no obligation to participate we do appreciate your time and effort. All information is kept secure and confidential under the Board of Directors of the Carol Ann Foundation. The identifiable data on individual patients will not be given to any third party without any individual permission. If you have any questions or concerns please feel free to contact Mary Smith: (e-mail: mbs85705@yahoo.com) or you can contact Dr. Shunji Tomatsu (tomatsushunji@gmail.com), appointed as the International medical adviser by The CAF.

CONSENT AGREEMENT

MPS -IVA Registry Consent Form

If you wish to participate in the MPS - IVA Registry, you (or your child) must read and agree to this Consent Form..

The Purpose of this Registry Database

The purpose of this Registry website is to gather a database comprised of records of persons who suffer from Mucopolysaccharidosis IV type A (MPS - IVA), also known as Morquio Syndrome. The first purpose of the Registry database is to provide a complete, systematically organized body of clinical information about the disorder to help researchers understand the disorder and discover treatment and diagnostic approaches to alleviate or cure the disorder. The second purpose of the Registry database is to inform Morquio patients and interested parties as to the symptoms and time frames that may be helpful in understanding this disorder further.

What We Collect:

We collect personal demographic information about each Morquio patient as well as a detailed medical history of each person by asking him or her (or their parents) to complete the IMO Profile Questionnaire. The questionnaire information is collected via the form on the website, or via the postal system. You may print out this questionnaire and complete it offline.

Please send to:
The Carol Ann Foundation
8164 W. Circulo De Los Morteros
Tucson, AZ 85743

WE DO NOT COLLECT ANY INFORMATION DIRECTLY FROM PERSONS UNDER THE AGE OF 18 YEARS. WE ONLY COLLECT SUCH INFORMATION ABOUT THOSE UNDER 18 YEARS OF AGE THROUGH THE DIRECT PARTICIPATION OF THEIR PARENTS OR LEGAL GUARDIAN.

What We Do With the Information We Collect:
The information that the Carol Ann Foundation collects about the Morquio patient is entered into the Registry database in a standard format ("record") and stored in a secure server. Only CAF personnel and Dr. Tomatsu is allowed access to this information. Dr. Tomatsu is compiling, summarizing and analyzing this information for research purposes. The CAF will provide access to this summarized analyst in the future via its own web site and the CAF newsletter.

The staff of CAF who collect the information to enter into the Registry database will know your (or your child's) name and other unique identifying information. These persons are obligated to keep this information confidential and to handle the information gathered about the Morquio patient is kept with the utmost attention to maintaining the confidentiality of the people involved. The Registry database will be stored on a secure server owned and operated by the CAF. The CAF has undertaken the responsibility to not access individual, identifiable patient records or information collected on the Registry Database except when necessary to ensure the integrity and utility of the Database. The CAF has agreed not to use or disclose the individual, identifiable data for any reason except as specifically stated in these Terms and Conditions.

The CAF Board of Directors will review the protocol of the questionnaire, the registry, and database procedures to ensure patient confidentiality.

Other Times When the Information Might Be Released:

Information collected through this website will only be released to bona fide researchers as described above, or if we are required by law to release the information.

How We Keep Information Secure:

The registry information is kept on a secure server with access limited as mentioned above. Periodically a copy of this information is transferred to a secure maximum security offsite storage facility. These security measures, we do not warrant, represent or guarantee that our security will not fail or be breached.

PLEASE READ THE FOLLOWING STATEMENTS CAREFULLY BEFORE SIGNING THIS CONSENT AGREEMENT

I understand that my (or my child's) identity will not be disclosed except to those persons who are responsible for compiling, verifying and maintaining the information to be stored in the Registry database or except as I may consent in writing.

I understand that despite reasonable security precautions there is a risk that my (or my child's) identity and other personal information could be inadvertently revealed. This could happen by interception or misdirection of electronic communications, penetration of security systems by unauthorized users ("hackers"), theft or misappropriation of the key linking names to numbers and similar mishaps.

I understand that the data I (or my child) provide may be used in scientific publications, analyzed as a group only, not individually.

I understand that the data I (or my child) provide may be viewed by researchers with Institutional Review Board (IRB) or Ethics Committee (EC) approved studies on MPS -IVA. Individual records without individual identifying information (your name, address, telephone number, etc.) would be made available to these researchers. Information about your (or your child's) physician information would be made available to these researchers.

I understand that there may be no direct benefit to me (or my child) through participation in the Registry, but that findings may provide valuable information about MPS -IVA and its treatment.

I understand by participating in the Registry, I (or my child) may be notified of research studies in which I (or my child) may be eligible to participate.

I understand that I (or my child) will not be excluded from future studies or suffer any other consequence if I (or my child) decline to participate in the Registry.

I understand that my (or my child's) participation in the Registry is voluntary and free of cost to me (or my child). I

understand that to participate in the Registry, I must authorize the release of my (or my child's) medical information by my (or my child's) physician for the sole purpose of completing and verifying my (or my child's) medical condition for inclusion in the Registry.

I understand that I can withdraw from the Registry at any time by logging in to www.morquio.com/Withdrawl 092602.pdf, printing out the Withdrawal form and mailing it to the Registry Coordinator.

I agree to the Terms and Conditions.

I consent to allow information about me (or my child) to be entered in the Registry as described in the statements in this Consent Form.

I agree that by clicking this form I acknowledge that I have read and understand the statements above. I

AM 18 YEARS OF AGE OR OLDER. If you are not yet 18, this form must be signed by your parent or legal guardian.

AGREE DISAGREE

Agree: ☐ **Disagree:** ☐

First Name: ______ Last Name: ______ M.I. ______

Date: ______

ANNUAL QUESTIONAIRE

THIS ANNUAL QUESTIONAIRE IS FOR ALL MORQUIO PATIENTS. THIS IS TO KEEP OUR DATABASE UPDATEDWITH THE MOST CURRENT MEDICAL INFORMATION ON EACH PATIENT. IF YOU ARE UNABLE TO SAVE THIS FILE, PLEASE COPY ANY PASTE IT INTO MICROSOFT WORD, SAVE IT AND SEND IT TO: mbs85705@yahoo.com THANK YOU.

Date: ______

Patients Name: ______

DATE OF BIRTH: ______

Sex:
Male ☐
Female ☐

Birth place(city state country): ______

Race
- ☐ Caucasian/White
- ☐ Native Hawaiian or other Pacific Islander
- ☐ American Indian or Alaska Native
- ☐ Black or African American
- ☐ Asian

Other

Ethnicity:
- ☐ **Hispanic/Latino**
- ☐ **Not Hispanic/Latino**

Birth weight: (kg, or pounds)

Birth Height: (cm or foot and inch)

Gestation:

Complications during pregnancy and/or birth:

Current weight: (kg, or pounds)

Current height: (cm or foot and inch)

Father:

Specify origin of country:

Present age Date of birth

Current Weight: (kg, or pounds) Current Height (cm or foot and inch)

Mother:

Specify origin of country:

Present age Date of birth

Current Weight: (kg, or pounds) Current Height (cm or foot and inch)

Patient'sbrothersandsisters:

1. Date of birth Sex: Male ☐ Female ☐
2. Date of birth Sex: Male ☐ Female ☐
3. Date of birth Sex: Male ☐ Female ☐
4. Date of birth Sex: Male ☐ Female ☐
5. Date of birth Sex: Male ☐ Female ☐

Present address:

Street: City:

State: Zip code:

Home Telephone number:

WORK Telephone number:

Fax: E-Mail:

Please fill in your growth chart referring to an example. Specifying cm or foot and inch (or you may send the growth chart by mail).

Height & Weight Chart

Age	1 year	2 year	3 year	4 year	5 year	6 year	7 year	8 year	9 year
Height **Check** **centimeters**									
or **Feet & Inches**									
Weight **Check** **kg**									
or **pounds**									

Height (cm or foot and inch) Weight (kg or pounds)

Age	10 year	11 year	12 year	13 year	14 year	15 year	16year	17 year	18 year
Height									
Weight									

Example:

Age	1 year	2 year	3 year	4 year	5 year	6 year	7 year	8 year	9 year
Height cm	70	75	100	110	119	125	129	135	140
Weight kg	10	11	12	15	18	21	25	28	30

Age	10 year	11 year	12 year	13year	14 year	15 year	16year	17 year	18 year
Height cm	150	155	160	165	170	175	180	181	180
Weight kg		40	45	50	55	60	65	70	70

1. List Surgeries:

- **Surgery: shunt placements** — **Date:**
- **Surgery: ear tubes placed** — **Date:**
- **Surgery: tracheotomy** — **Date:**
- **Surgery: tonsillectomy** — **Date:**
- **Surgery: adenectomy** — **Date:**
- **Surgery: gastric tube placement** — **Date:**
- **Surgery: hernia repair** — **Date:**

List Orthopedic Surgery:

- **hip replacement** — **Date:**
- **knee surgery** — **Date:**
- **leg surgery** — **Date:**

☐ **spine surgery** **Date:** ______

☐ **ankle surgery** **Date:** ______

☐ **cervical fusion** **Date:** ______

Specify other surgeries: Date: ______

2. WHAT KINDS OF MEDICATIONS OR ANY IS YOUR CHILD ON AT THIS POINT IN TIME?

CURRENT MEDICATION

Is the patient taking any prescription/OTC medication or herbal supplements or vitamins?
Yes ☐ No ☐

If yes, list the medications and the length of time over which each has been taken.

Brand Name	Indications	Date Started	Date Stopped

3. **WHO IS THE PRIMARY CARE PHYSICIAN THAT TAKES CARE OF YOUR CHILD?**
A. PLEASE LIST NAMES AND ADDRESS:

NAME:

HOSPITAL:

WORKING OFFICE PHONE:

FAX: ______

ADDRESS: ______

STREET: ______ CITY: ______

STATE: ______ ZIP CODE: ______

B. PLEASE LIST ALL SPECIALISTS INCLUDING ADDRESSES:

1. NAME:
HOSPITAL:
WORK TELEPHONE: **FAX:**
STREET ADDRESS:
CITY:
STATE: **ZIP CODE:**

2. NAME:
HOSPITAL:
WORK TELEPHONE: **FAX:**
STREET ADDRESS:
CITY:
STATE: **ZIP CODE:**

3. NAME:
HOSPITAL:
WORK TELEPHONE: **FAX:**
STREET ADDRESS:
CITY:
STATE: **ZIP CODE:**

4. When was your child diagnosed with Morquio's disease?

Specify Age (ex. 2 years and 1 month old etc.): ☐ Years ☐ Month old.

A. up to 1 year

B. 1 to 3 years

C. 3 to 5 years

D. 5 to 10 years

E. over 10 years

5A. Does your Geneticist follow and chart your CHILD'S GROWTH?
Yes ☐ No ☐

5B. At which age did growth stop?

6A. How was your child diagnosed with Morquio A syndrome (MPS IVA)? (Check the ones that apply)

1. Urine test (MPS screening test) ☐
2. Blood test (enzyme activity) ☐
3. Clinical physical examination ☐
4. Others (skin biopsy) ☐
5. X-rays ☐
6. Bone marrow biopsy ☐

OTHER Procedures and Tests:

Has the patient ever had any of the following procedures or test

	Procedure	Yes	Date
a.	Cerebral MRI		
b.	EEG		
c.	Sleep Study		
d.	Nerve Conduction Test		
e.	Abdominal MRI		
f.	Opthalmologic Exam		
g.	Skeletal X-rays		
h.	Hearing Test		
i.	Pulmonary function test		
j.	Physical Therapy		
	Type of Therapy		
k.	Joint range of motion		

6B. If your child received the urine, blood, or fibroblast test, please describe the results.

A. Urine:
Total GAG assay:

Keratan sulfate assay:

Other Assay:

B. Blood:
Enzyme activity:

Total GAG assay:

Keratan sulfate assay:

C. Fibroblast test
Enzyme activity of Morquio A enzyme:

7A. Has your child gotten DNA test of Morquio gene? (Check one)

1. yes ☐
2. no ☐
3. unknown ☐
4. recommended by doctor but not done ☐

B. If available, the mutation site of Morquio A :

8. When did you notice your child's clinical symptoms?

Specifyage (2 years old, and 1 month old): ☐ Years ☐ Months Old.

1. up to 1 year ☐
2. 1 to 3 years ☐
3. 3 to 5 years ☐
4. 5 to 10 years ☐
5. over 10 years ☐
6. never noticed anything ☐

9. What kinds of symptoms did your child have initially? (Check one or more)

1. bone deformity ☐

- **a.** KNEE (knocked Knee) ☐
- b. ANKLES (dislocated etc.) ☐
- c. FEET -LEG, TOES (curved, bowed, deformed, flat etc.) ☐
- d. SHOULDERS (curved, bowed, deformed, dislocated etc.) ☐
- e. HIPS (dislocated, deformed, etc.) ☐
- f. WRISTS (curved, bowed, deformed, dislocated etc.) ☐
- g. BACK - SPINE: Kyposis (hump back), Scoliosis, Lodorsis ☐
- h. CHEST - pigeon chest (curved, bowed, deformed etc.) ☐
- i. FINGERS (curved, bowed, deformed, dislocated etc.) ☐

2. SHORT STATURE (GROWTH RETARDATION) ☐

3. ABNORMAL GAIT ☐

4. DIFFICULTY OF JOINT MOVEMENT ☐

5. CERVICAL SPINE INSTABILITY ☐

6. HEARING LOSS ☐

7. RESTRICTION OF EFFICIENT BREATHING ☐

8. LEG, HIP, BACK PAIN ☐

9. LIVER ENLARGEMENT ☐

10. HERNIA ☐

11. EYE PROBLEMS (CORNEAL CLOUDING, DECREASED VISUAL POWER ETC.) ☐

12. CHRONIC EAR INFECTIONS ☐

13. INCREASED CRANIAL CIRCUMFERENCE (PROMINENT FOREHEAD) ☐

14. HYDROPS FETALIS (ONE OF COMPLICATIONS AT BIRTH) ☐

15. ABNORMAL FACES (COURSE FACE) ☐

16. SHORT NECK ☐

17. HEART DISEASE (VALVE DISEASE, ARRHYTHMIA ETC.) ☐

18. TEETH PROBLEMS (THIN ENAMEL, SMALL AND WIDELY SPACED TEETH, FREQUENT CARIES ETC.) ☐

19. STOMACH PROBLEMS(VOMITING, NAUSEA ETC.) ☐

20. RECURRENT RESPIRATORY INFECTION ☐

21. SNORING ☐

22. TIRES EASILY (STAMINA) ☐

23. LAXITY OF JOINT(S) - FINGER, ELBOW, KNEE, HIP, WRIST (DOUBLE JOINT, LOOSE LIGAMENT ETC.) ☐

24. FALLS FREQUENTLY ☐

25. DIFFICULTY OF HANDWRITING ☐

26. others (specify):

10. What kinds of symptoms does your child have right now? (check one or more)

1. bone deformity ☐

   a. KNEE (knocked Knee) ☐
   b. ANKLES (dislocated etc.) ☐
   c. FEET -LEG, TOES (curved, bowed, deformed, flat etc.) ☐
   d. SHOULDERS (curved, bowed, deformed, dislocated etc.) ☐
   e. HIPS (dislocated, deformed, etc.) ☐
   f. WRISTS (curved, bowed, deformed, dislocated etc.) ☐
   g. BACK - SPINE: Kyposis (hump back), Scoliosis, Lodorsis ☐
   h. CHEST - pigeon chest (curved, bowed, deformed etc.) ☐
   i. FINGERS (curved, bowed, deformed, dislocated etc.) ☐

2. SHORT STATURE (GROWTH RETARDATION) ☐

3. ABNORMAL GAIT ☐

4. DIFFICULTY OF JOINT MOVEMENT ☐

5. CERVICAL SPINE INSTABILITY ☐

6. HEARING LOSS ☐

7. RESTRICTION OF EFFICIENT BREATHING ☐

8. LEG, HIP, BACK PAIN ☐

9. LIVER ENLARGEMENT ☐

10. HERNIA ☐

11. EYE PROBLEMS (CORNEAL CLOUDING, DECREASED VISUAL POWER ETC.) ☐

12. CHRONIC EAR INFECTIONS ☐

13. INCREASED CRANIAL CIRCUMFERENCE (PROMINENT FOREHEAD) ☐

14. HYDROPS FETALIS (ONE OF COMPLICATIONS AT BIRTH) ☐

15. ABNORMAL FACES (COURSE FACE) ☐

16. SHORT NECK ☐

17. HEART DISEASE (VALVE DISEASE, ARRHYTHMIA ETC.) ☐

18. TEETH PROBLEMS (THIN ENAMEL, SMALL AND WIDELY SPACED TEETH, FREQUENT CARIES ETC.) ☐

19. STOMACH PROBLEMS(VOMITING, NAUSEA ETC.) ☐

20. RECURRENT RESPIRATORY INFECTION ☐

21. SNORING ☐

22. TIRES EASILY (STAMINA) ☐

23. LAXITY OF JOINT(S) - FINGER, ELBOW, KNEE, HIP, WRIST (DOUBLE JOINT, LOOSE LIGAMENT ETC.) ☐

24. FALLS FREQUENTLY ☐

25. DIFFICULTY OF HANDWRITING ☐

26. others (specify):

11. How is your child's intelligence? (Check one or more)

1. excellent ☐
2. normal ☐
3. poor ☐
4. more retarded than before ☐
5. hard to learn because of disease ☐

12. Do you think your child's clinical course is getting worse or not? (check one)

1. RAPIDLY WORSE ☐
2. SLOWLY WORSE ☐
3. STABLE ☐
4. IMPROVING ☐

13. How much does your child take care alone?

1. How far is your child currently able to walk without any assistance. Please specify:

A. Less than 1 block (0-200 meters: 0-1/8 mile) ☐
B. Less than 2 blocks (200-400 meters: 1/8-1/4 mile) ☐
C. Less than 1/2 mile (400-800 meters: 1/4-1/2 mile) ☐
D. More than 1/2 mile (over 800 meters: over 1/2 mile) ☐

2. Does your child need a cane or walker to get around? Walks with an aid (please specify):

A. Scooter ☐
B. Walker ☐
C. Cane ☐

3. Do you need a Wheelchair to get around? ☐

Specify what age:

4. Bedridden (please give details):

5. Has your child ever worn a brace? yes ☐

A. Specify type:
B. What age:
C. Length of time:

Is it effective or not?

a: very effective ☐
b. moderately ☐
c. slightly ☐
d. no change ☐
e. worse ☐
f. do not like to wear or hard to wear

6. Rides a bicycle alone? Yes ☐

7. Takes a bath alone? Yes ☐

8. Puts clothes on alone? Yes ☐

9. Takes clothes off alone? Yes ☐

10. Can your child stand on their own? Yes ☐

11. Favorite activities (check one or more):

a. Swim ☐
b. Plays with blocks ☐
c. Dolls ☐
d. Basketball, Baseball ☐
e. Soccer ☐
f. Reading ☐
g. Fishing ☐
h. Computer ☐
i. Play instrument (Piano, Flute, Guitar etc.) ☐
j. Drawing, writing, and handicraft ☐
k. Play videogames ☐
l. Others: Specify

12. Lives and works independently ☐

13. Fine Motor Skills (Specify)

a. Buttons ☐
b. Zippers ☐
c. Snaps ☐
d. Puzzles ☐
e. Others: Specify

14. Are you able to drive a vehicle that is specially equipped? Yes ☐

15. Has your child ever been to any physically challenged camps? (specify)

16. Are you involved in any clubs or organizations?

14. PHYSICAL STATUS

***Severity scale: 1-Mild 2-Moderate 3-Severe**

		Yes	Age Onset	Severity scale
	CARDIOVASCULAR			
1.	Systemic hypertension	☐		
2.	Cardiomyopathy	☐		
3.	Conduction abnormality or Arrhythmia	☐		
4.	Congestive heart failure	☐		
5.	Valve Disease	☐		
6.	Angina	☐		
	EYES			
7.	Retinal degeneration	☐		
8.	Corneal pathology	☐		
9.	Lens pathology	☐		
10.	Decreased visual acuity	☐		
11.	Optic nerve atrophy	☐		
12.	Papilledema	☐		
	Ear, Nose, Throat			
13.	Tinnitus	☐		
14.	Hearing Loss - Conductive	☐		
15.	Hearing Loss - Sensory/Neuro	☐		
16.	Vertigo	☐		
17.	Ortitis Media (frequency of infection)	☐		
18.	Swallowing Difficulties	☐		
19.	Rhinorrhea	☐		
20.	Delayed Dentition	☐		
21.	Abnormal Dentition	☐		
22.	Dental Abcess	☐		
23.	Gingival Hypertrophy	☐		
24.	Enlarged Tongue	☐		
25.	Enlarged Tonsils	☐		
26.	Enlarged Adenoids	☐		
	HEPATIC			

27.	Hepatomegaly	☐		
28.	Splenomegaly	☐		
	GASTROINTESTINAL			
29.	Abdominal pain	☐		
30.	Diarrhea	☐		
31.	Bowel Incontinence	☐		
32.	Weight Loss	☐		
33.	Constipation	☐		
34.	Enema Dependent	☐		
35.	Toilet Trained No	No ☐		
36.	If #35 is no, were they trained and later lost this control?	Yes ☐		
	***Severity Scale: 1-Mild 2-Moderate 3-Severe**	**YES**	**AGE ONSET**	**SEVERITY**
	GENITOURINARY			
37.	Bedwetting	☐		
38.	Diapers required during day	☐		
39.	Diapers required during night	☐		
40.	UTI	☐		
	MUSCULOSKELETAL			
41.	Joint Pain	☐		
42.	Joint Laxity	☐		
43.	Joint Stiffness	☐		
44.	Cervical Myelopathy	☐		
45.	Cervical Sublaxation	☐		
46.	Cord Compression	☐		
47.	Scoliosis	☐		
48.	Kyphosis	☐		
49.	Gibbus	☐		
50.	Knocked Knee	☐		
51.	Dislocated Hips	☐		
52.	Hip Dysplasia	☐		
53.	Clawed Hands	☐		
54.	Carpal Tunnel Syndrome	☐		
	NEUROLOGICAL			
55.	Nerve Entrapment	☐		
56.	Hydrocephalus	☐		
57.	Seizure Disorder	☐		
58.	Transient Ischemic Attacks	☐		
59.	Stroke	☐		
60.	Mental Retardation	☐		
	PSYCHIATRIC			

		YES OR NO	ONSET YEAR	SEVERITY(1-3)
61.	Anxiety Disorders	☐		
62.	Depression	☐		
63.	Hyperactivity	☐		
64.	Agressive Behavior	☐		
	SKIN			
65.	Papular Lesions	☐		
66.	Skin Breakdown/Lesions	☐		
67.	Atopic Dermatitis	☐		

15. The Most Important Symptom is Respiratory-Related. Please Check It The Following Respiratory Issue.

***Severity scale 1-Mild 2-Moderate 3-Severe**

	RESPIRATORY	YES OR NO	ONSET YEAR	SEVERITY(1-3)
1.	Oxygen Dependent	☐		
2.	Obstructive Airway Disease	☐		
3.	Asthmatic	☐		
4.	Chronic Bronchitis	☐		
5.	Sleep Apnea	☐		
6.	Narrowed Trachea	☐		
7.	Pneumonia	☐		

16. What is the most serious problem with your child right now? (check the ones that apply)

1. Growth Retardation ☐
2. Weakness of Wrist ☐
3. Lower Back Pain ☐
4. Hip Pain ☐
5. Stomach Problems (Vomiting, nausea, pain etc.) ☐
6. Knee (knocked knee) Pain ☐
7. Chest (pigeon chest, restrictive etc.) ☐
8. Bone Deformity ☐
9. Central Nervous System (paraplegia) ☐
10. Difficulty to climb stairs ☐
11. Severe Constipation ☐
12. Risk of injury due to cervical instability ☐
13. Infections (ear, respiratory) ☐
14. Osteoporosis ☐
15. Leg Pain ☐
16. Inability to walk ☐
17. Inability to work (lack of mobility or limited mobility) ☐
18. Breathing Difficulties ☐
19. Feet Turn inward ☐
20. Falls Frequently ☐
21. "Lax" Elbow and Wrist Joints Becoming "Unhinged" ☐
22. Emotionally Hard ☐
23. Apnea ☐
24. Weight Problem ☐
25. Stamina (tires easily, physically weak) ☐
26. Hearing Loss ☐
27. Loss of Visual Power ☐

28. Please Write Without Hesitation On Any Issue From Medical Problems to Daily Care:

17. If Enzyme Replacement Therapy Has Been Developed, Are You Willing To Have The Opportunity To Participate In A Clinical Trial?

1. Yes ☐
2. No ☐
3. Unknown ☐
4. If We Understand Enzyme Replacement Therapy, We Will ☐

18. Normally, to develop diagnostic system, advanced treatments (Enzyme Replacement Therapy etc.)and other research related on Morquio A Syndrome (MPS IVA), a lot of funding will be necessary. Would you agree to work with the Carol Ann Foundation to raise the fund? (please go to the web site link under "funding" for more details).

1. Yes ☐
2. No ☐
3. Unknown ☐
4. If we understand MPS IVA related research in more details, we agree. ☐

19. What type of clinical manifestations or clinical course of Morquio Type A disease does your child have?

1. Classical (severe) ☐
2. Intermediate ☐
3. Mild ☐
4. Unknown ☐
5. Between Intermediate and Severe ☐
6. Some Areas (for example: spine) - Considered Severe - Though Diagnosed as Mild ☐
7. Other (Specify):

20. What Do You Expect of Us Scientists?

1. Risk worry (REGARDING ENZYME REPLACEMENT TREATMENT) ☐
2. Would you like to know the cause of Morquio Type A and to have more information available ☐
3. To find a cure ☐
4. To respect privacies of families ☐
5. To give everyone frequent updates ☐
6. Treat animals humanely ☐
7. To ease disease progression ☐
8. To be able to diagnose quicker ☐
9. Help address the issues that concern older patients ☐
10. Increase growth ☐
11. To have a normal life span ☐
12. To be able to avoid surgeries altogether ☐
13. To help growing pains ☐
14. To help increase activity and stamina ☐

21. If you want to have Dr. Tomatsu contact you, please let me know so that we can arrange a schedule.

1. Yes ☐
2. No ☐

22. Please let me know if you need an educational CD for Morquio.

1. Yes ☐
2. No ☐

THANK YOU FOR YOUR PARTICIPATION

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