

MPS I Disease Management Patient Survey

A collaboration of Genzyme and the National MPS Society

January 2007

Contents

- ☐ Objectives & Methodology
- ☐ Current Health
- ☐ Transplantation
- ☐ Enzyme Replacement Therapy
- ☐ Support & Services
- ☐ Summary

Objectives

- ❑ **Ask MPS I patients about their current health, both physical and emotional**
- ❑ **Examine their experience of transplantation and enzyme replacement therapy, and how well these therapies lived up to patient expectations**
- ❑ **Ensure optimal treatment across patient groups, i.e., the spectrum of MPS I disease (Severe and Attenuated), and organ systems**
- ❑ **Obtain input on patient support services provided by Genzyme and the National MPS Society in order to plan programs that encourage optimal treatment**

Methodology

- ❑ **8-page questionnaire designed by Genzyme in collaboration with National MPS Society**
- ❑ **Society mailed survey package**
 - **Questionnaire, cover letter from Society director, stamped reply envelope to 136 households with 149 patients**
- ❑ **Survey period**
 - **September-October 2006**
- ❑ **Survey response**
 - **61 completed questionnaires returned (41% response rate)**

**RESPONDENT
DEMOGRAPHICS**

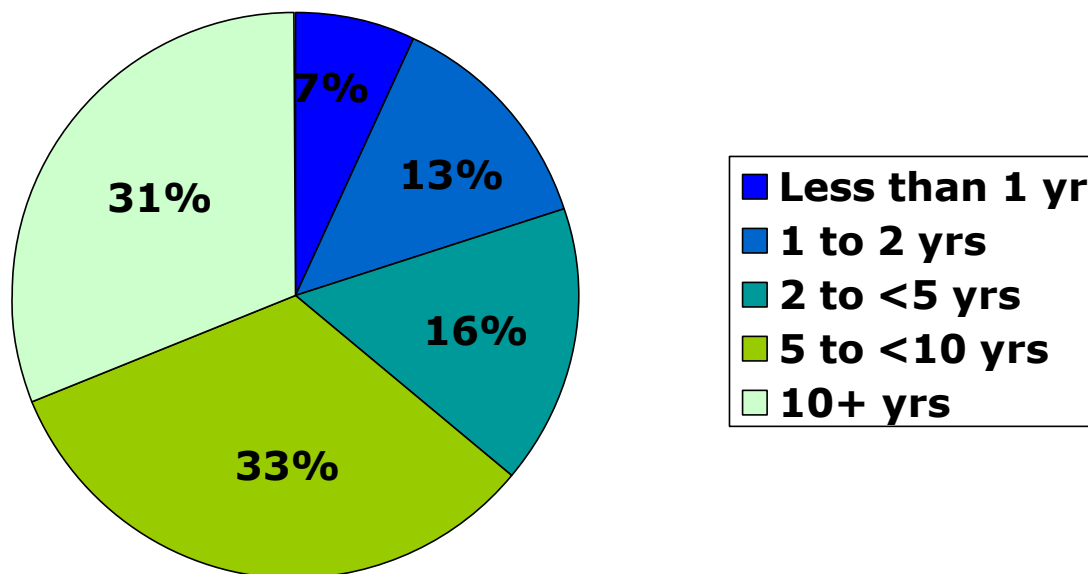
The survey achieved good representation across patient segments

- ❑ **Respondent is:** patient (18%), parent/guardian (82%)
- ❑ **Patient gender:** male (50%), female (50%)
- ❑ **Patient age:** <6 years (25%), 6<18 (53%), 18+ (22%)
- ❑ **Disease severity:** Severe (66%), Attenuated (34%)
- ❑ **Transplantation recipient (54%)**
- ❑ **Enzyme Replacement Therapy recipient: (49%) including current (36%, including 15% on for <3 yrs and 21% on for 3+ yrs) and past (13%, e.g., short-term during/after transplantation)**
- ❑ **Insurance:** all have health insurance; 80% have private insurance and 38% have Medicaid (indicating that many privately insured also have Medicaid assistance)
- ❑ **Geography:** 40% South, 25% West, 23% Midwest, 12% Northeast

RESPONDENT DEMOGRAPHICS

Respondents include newly diagnosed patients, as well as patients diagnosed many years ago before new therapies were available

How Long Ago Diagnosed



Q2. Approximately, how long ago were you first diagnosed with MPS I disease? (N=61)

RESPONDENT DEMOGRAPHICS

1/3 of primary managers of MPS I disease are geneticists and transplant specialists, while many are non-experts

Specialty of “Primary Manager” of MPS I

“Expert” specialties

Geneticist	25%
Transplant specialist	11%

Non-experts

Pediatrician	20%
Hem/onc	13%
GP, internal, PCP	12%
Don't have “primary manager”	7%
Other/Multiple specialties	12%

RESPONDENT DEMOGRAPHICS

Many patients see multiple physician specialists for symptom management

Other Physicians Seen Regularly (at least once a year)

Cardiologist	80%
Ophthalmologist	76%
Dentist	68%
Orthopedic surgeon	63%
Ear-nose-throat (ENT)	61%
Pediatrician	51%
Physical therapist	49%
Geneticist	46%
Occupational therapist	44%
Neurologist	42%
Hematologist/oncologist	39%
Pulmonologist	37%
GP, internal medicine, PCP	32%
Speech therapist	16%
Endocrinologist	10%
Gastroenterologist	9%
Transplant specialist	3%
Rheumatologist	--

Rheums may be
diagnosers, but are not
currently managing

Q7. Which of the following other physicians/medical professionals are you seeing on a regular basis, that is, at least once a year, for the management of MPS I? (N=61)

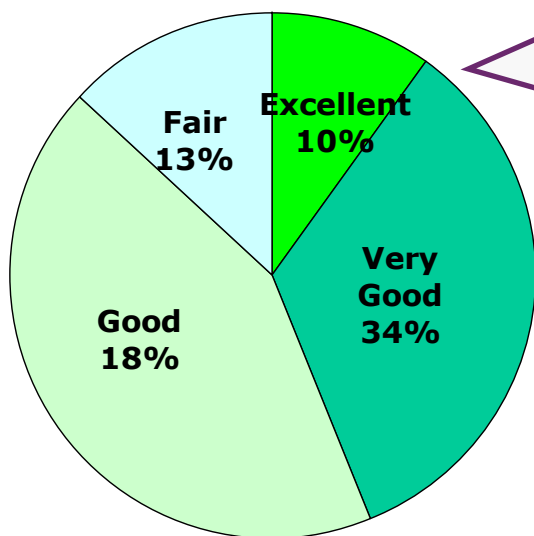
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Self-assessment of most MPS I patients' health is positive on overall physical and emotional measures

Overall Health Assessment

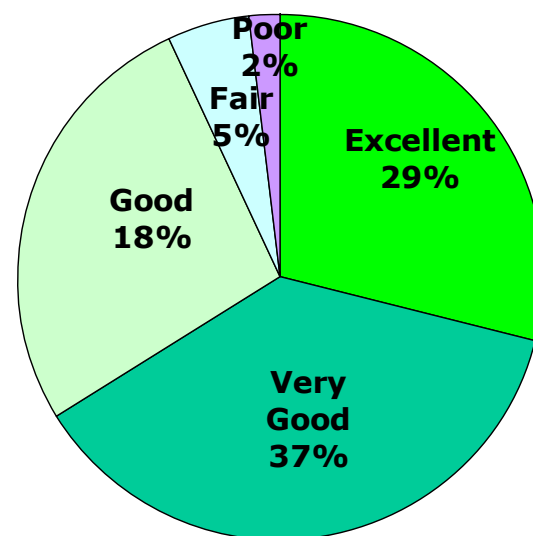
Physical Health



Higher **favorable** scores for Severe (50% Ex/VG) and age <6 (60%)

Higher **unfavorable** scores for Attenuated (24% Fair/Poor) and age 18+ (23%)

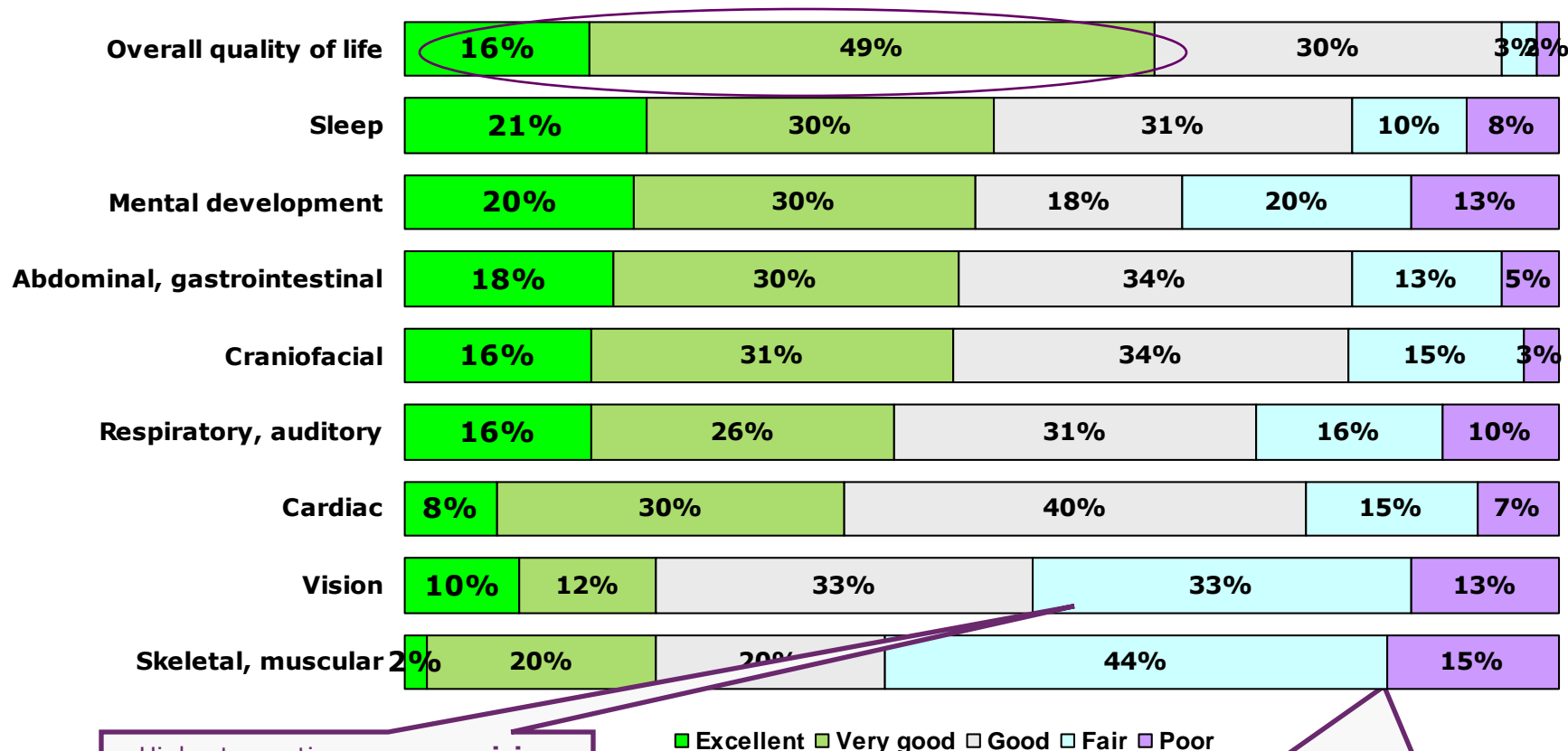
Emotional Health



Higher **unfavorable** scores for Attenuated (19% Fair/Poor)

Patients' highest health rating is on quality of life; on physical symptoms there are significant negative ratings, especially musculoskeletal and vision

Health Parameter Ratings



Highest negative scores on **vision** are from Severe (58% Fair/Poor) and 6<18 year olds (52%)

Highest negative scores on **musculoskeletal** are from 6<18 year olds (68% Fair/Poor), Severe (63%)

Q5. How would you rate your current health in each of these areas. . . ? (N=61)

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Transplantation Experience

Patients who have received transplantation

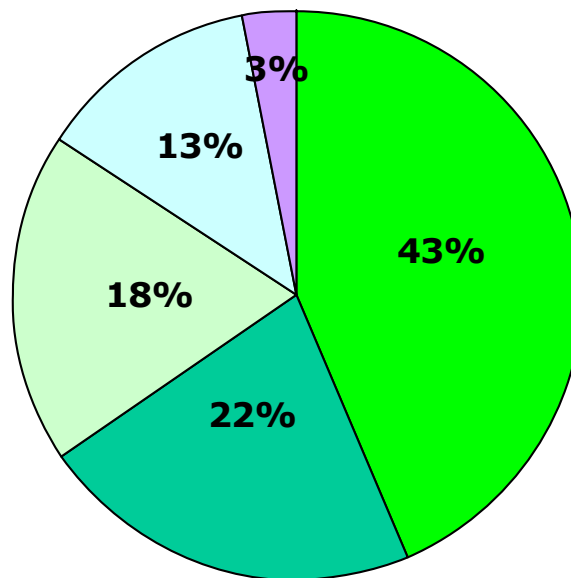
- ☐ **Phenotype**: 80% of Severe patients in this survey have received transplantation; 5% of Attenuated
- ☐ **Age** at transplantation: 1/3 before age 1; 2/3 at 1-2 years old
- ☐ **Type** of transplantation: 59% bone marrow, 38% umbilical cord blood, 3% peripheral blood
- ☐ 72% report having **side effects/complications**
- ☐ 22% report having **transplantation repeated**

Transplantation has met or exceeded most recipients' expectations; in many cases, far exceeding

How Has Transplantation Met Expectations?

Far exceeding expectations reported by:

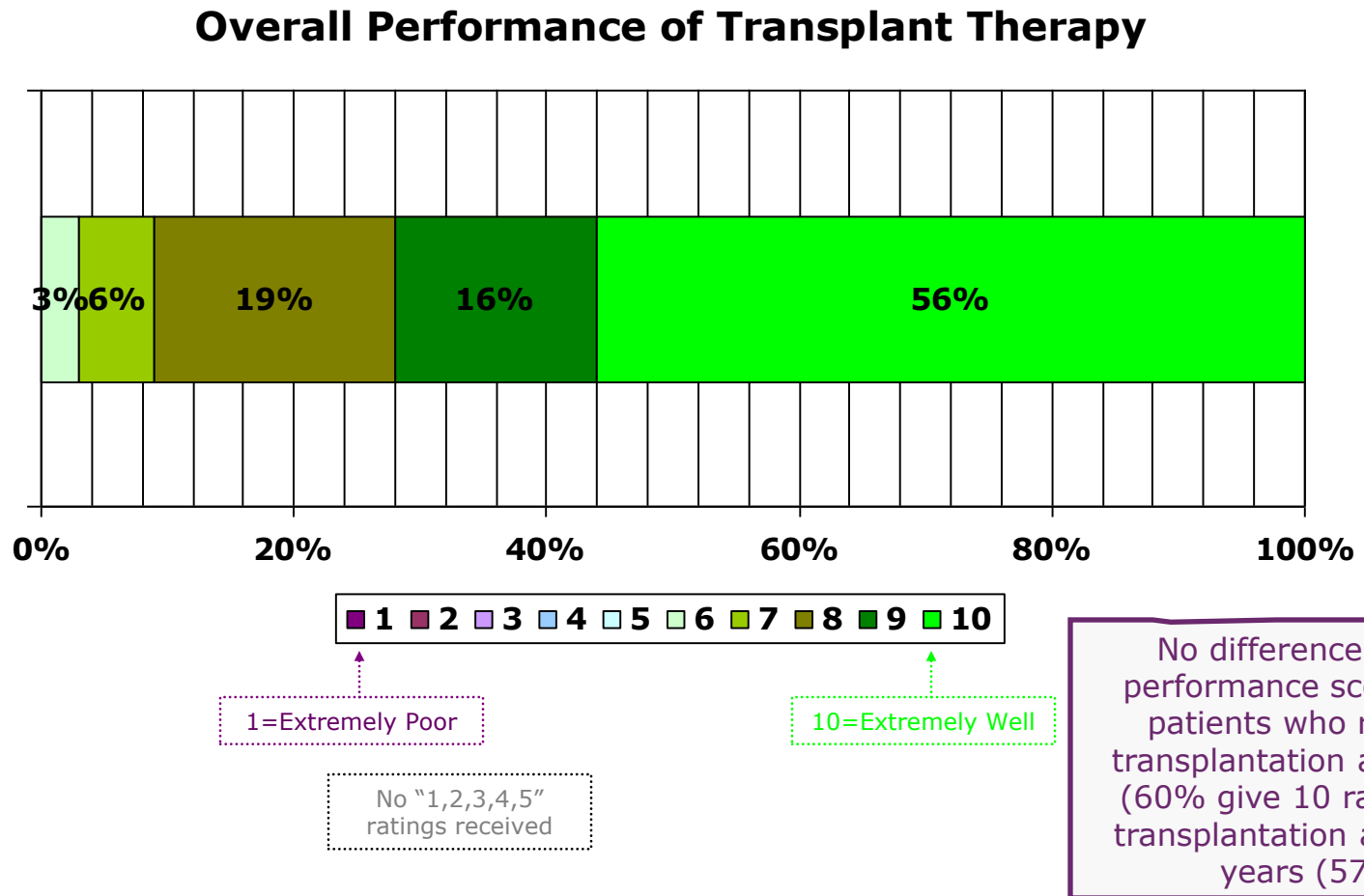
- 100% (1 patient) who received peripheral stem cell transplant
- 58% (7 of 12) of umbilical cord transplant patients
- 32% (6 of 19) of bone marrow transplant patients



■ Far exceeded
■ Slightly exceeded
■ Met expectations
■ Fell slightly short
■ Fell very short

More
disappointment
for age 6<18 (25%
fell short)

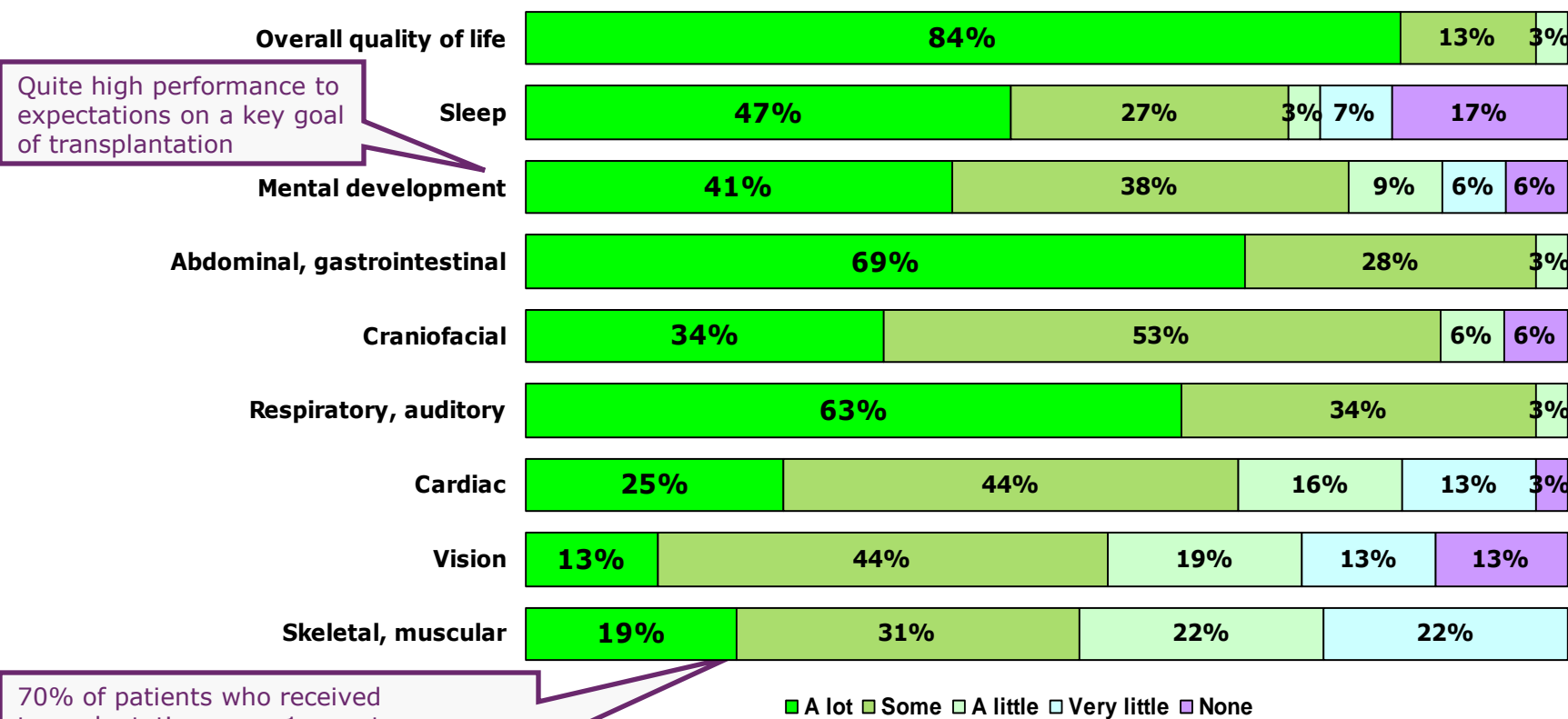
A majority of recipients rate transplantation performance a “10” on a 10-point scale



Q14. Based on your experience to date, how would you rate the overall performance of transplant therapy as a treatment for MPS I? (N=32)

Transplantation patients report “a lot” or “some” improvement on many parameters (including mental development), especially QoL, abdominal, respiratory; less on vision, musculoskeletal

Transplant Improvement on Health & Symptoms



Q15. How much improvement, if any, do you believe transplant therapy has had on your health and symptoms in each of these areas? (N=32)

When asked to comment on ways transplantation fell short or to suggest improvements, many patients focused on musculoskeletal & joint problems, followed by cognitive development, poor vision

Transplantation Shortcomings/Improvements Needed

Volunteered (multiple responses accepted)

Orthopedic/skeletal issues/kyphosis/bone structure	25%
Stiffness/joint pain/carpal tunnel/wanted to be more flexible	13%
Cognitive development/learning delay/mental retardation/speech	13%
Cloudy corneas/poor vision/want clearer corneas	13%
Growth delay	9%
Brain damage/brain disease	6%
Longer recovery time than anticipated	6%
Did not work as well as we hoped/fell short as a treatment	6%
Long term complications (unspecified)	3%
Cannot correct damage already occurred/does not repair	3%
Unexplainable drop in enzyme levels	3%
None	3%
All other	6%
None, N/A, No comment	34%

Musculo-skeletal issues widely mentioned for:
Male & <age 6

Cognitive issues mentioned widely for:
Female & age 6<18

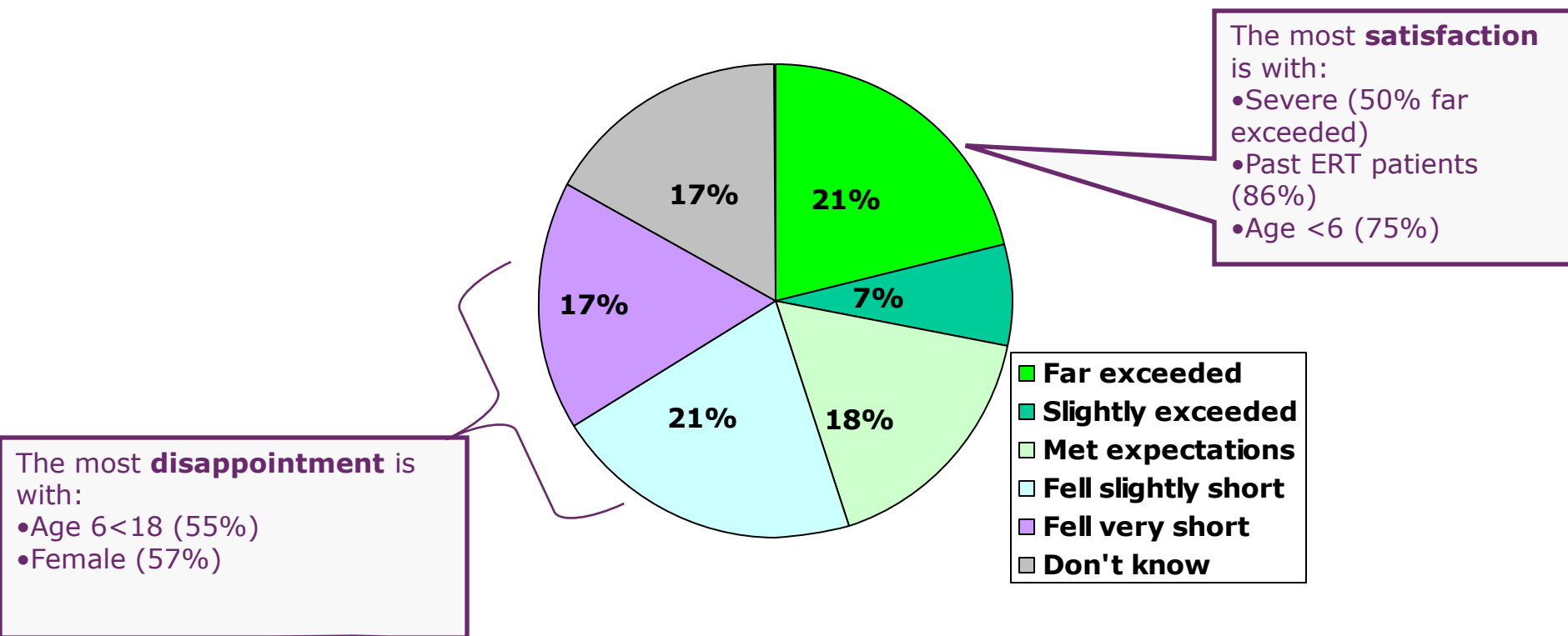
Q16. In what ways, if any, did transplant therapy fall short as a treatment, or what additional improvements would you want/expect the transplant to provide? (N=32)

Contents

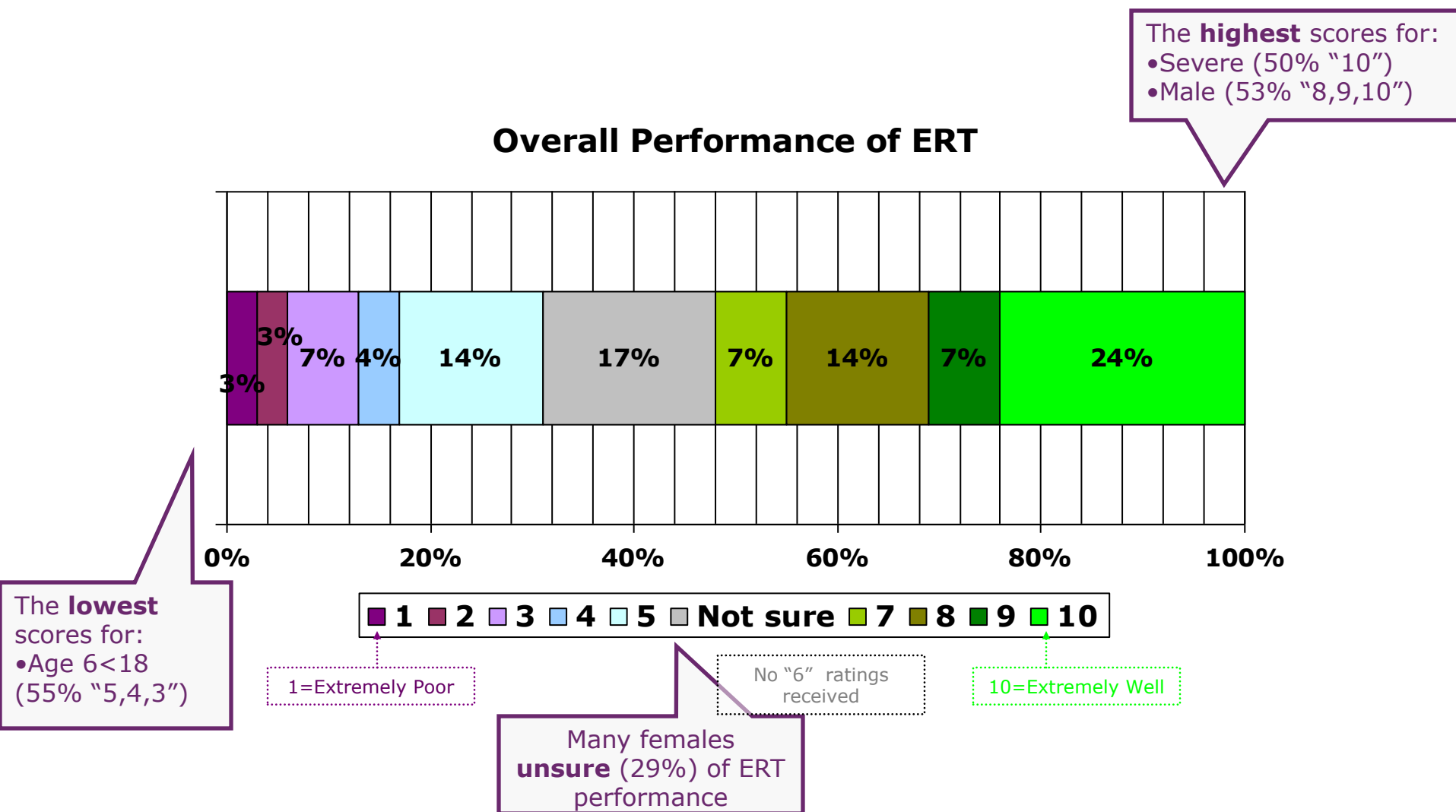
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Patients vary in their assessment of whether Enzyme Replacement Therapy has met or exceeded expectations

How Has ERT Met Expectations?



A 52% majority rate Enzyme Replacement Therapy performance favorably, while 1/3 give relatively low scores, and another 17% are unsure how to rate its performance

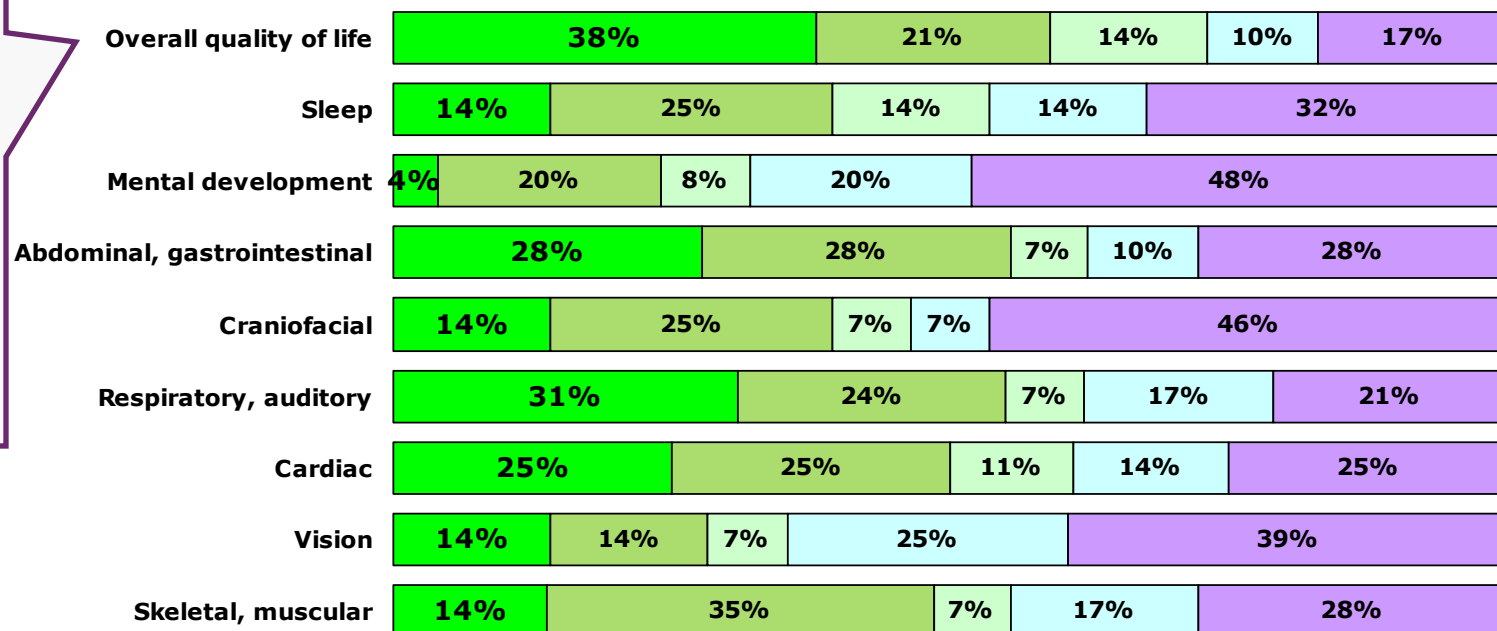


The most improvement from ERT reported on quality of life, abdominal, respiratory & cardiac parameters; least improvement reported on mental development, vision

ERT Improvement on Health & Symptoms

Widespread improvement reported on **QoL**:

- Age <6 yrs(88% Some/A lot), Age 6<18 (36%), Age 18+ (60%)
- Male (67%), Female (50%)
- Severe (70%), Attenuated (50%)



■ A lot ■ Some ■ A little ■ Very little ■ None

The **most** improvements across parameters reported:

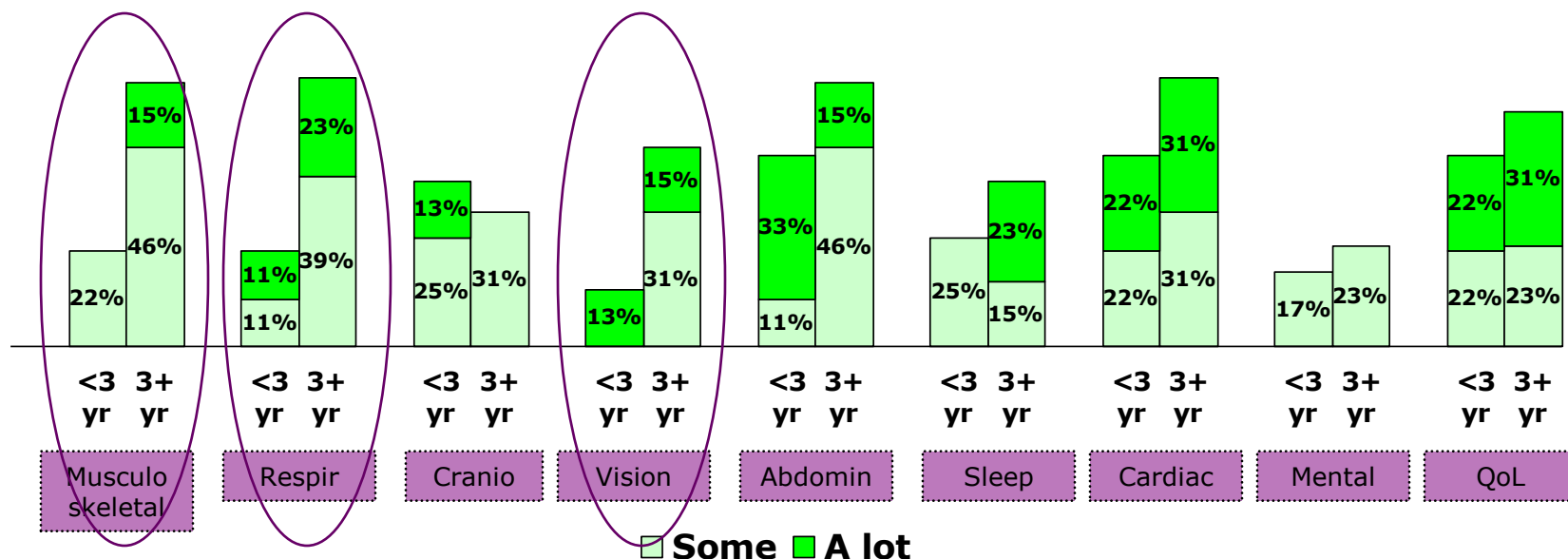
- Age <6 yrs
- Male
- Currently on ERT & been on 3+ yrs

The **least** improvements reported:

- Attenuated
- Age 6<18
- Female

The longer that patients have been on Enzyme Replacement Therapy, the better their reported outcomes, especially for respiratory, musculoskeletal and vision

Perceived Improvements from ERT, by Length of Time on ERT
(Current ERT patients)



Q25. How much improvement, if any, do you believe ERT has had on your health and symptoms in each of these areas?

(N=9 currently on ERT <3 yr; N=13 currently on ERT 3+ yr)

Patients are looking for greater improvements in musculoskeletal health, and reassurance that Enzyme Replacement Therapy at least stops disease progression; there is some understanding that therapy should start early

These parallel shortcomings mentioned regarding transplantation

Enzyme Replacement Therapy Shortcomings/ Improvements Needed

Volunteered (multiple response accepted)

Musculoskeletal

Skeletal issues

13%

Carpal tunnel/stiffness in joints/would like lessening of

7%

Growth

3%

Muscular symptoms

3%

Progression

Not seeing any help/have not noticed any changes/don't see any difference

10%

Has not stopped/slowed disease progression

7%

Does not reverse the disease/damage already caused

3%

Needs to be started younger

3%

Other

Pulmonary/problems with airway

3%

Wish it could break the brain barrier

3%

Fatigue/would like to improve stamina

3%

All other

7%

None, N/A, no comment

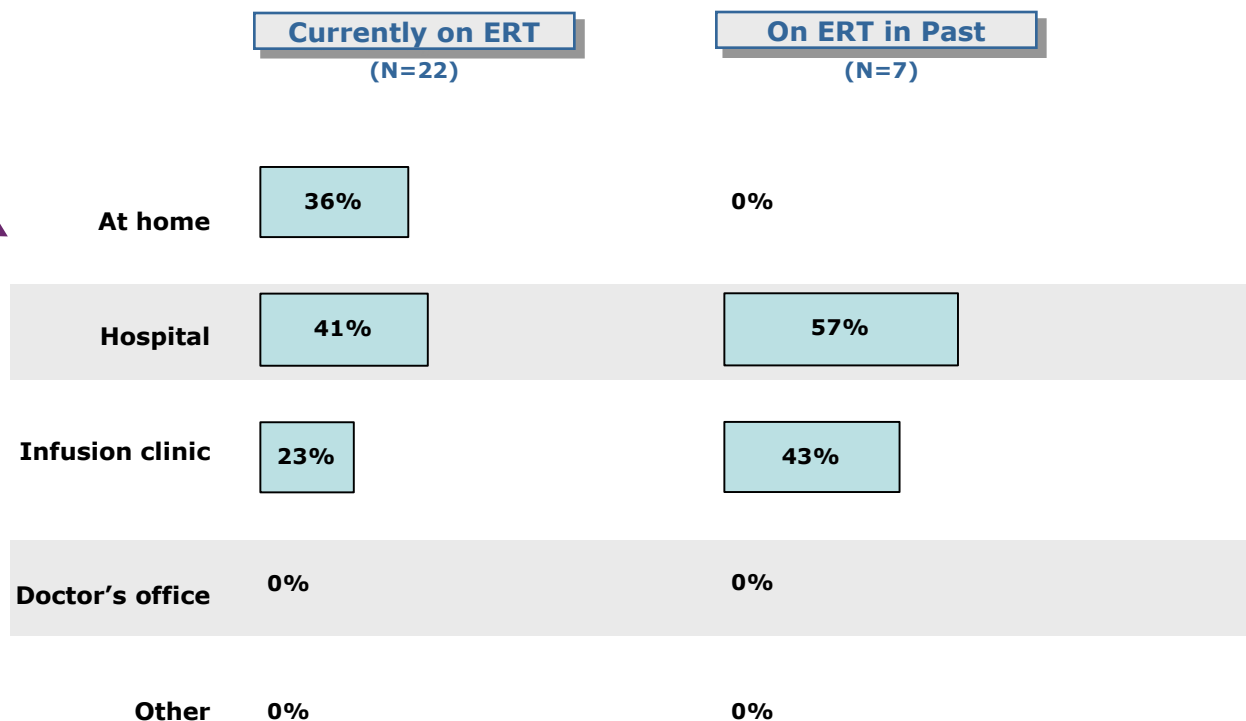
50%

Q26. And in what ways, if any, did ERT fall short as a treatment, or what additional improvements would you want/expect ERT to provide? (N=30)

ERT LOCATION

Many MPS I patients have adopted home infusion therapy, especially those age 6<18 and females, probably to lessen disruption to school/ work/ home

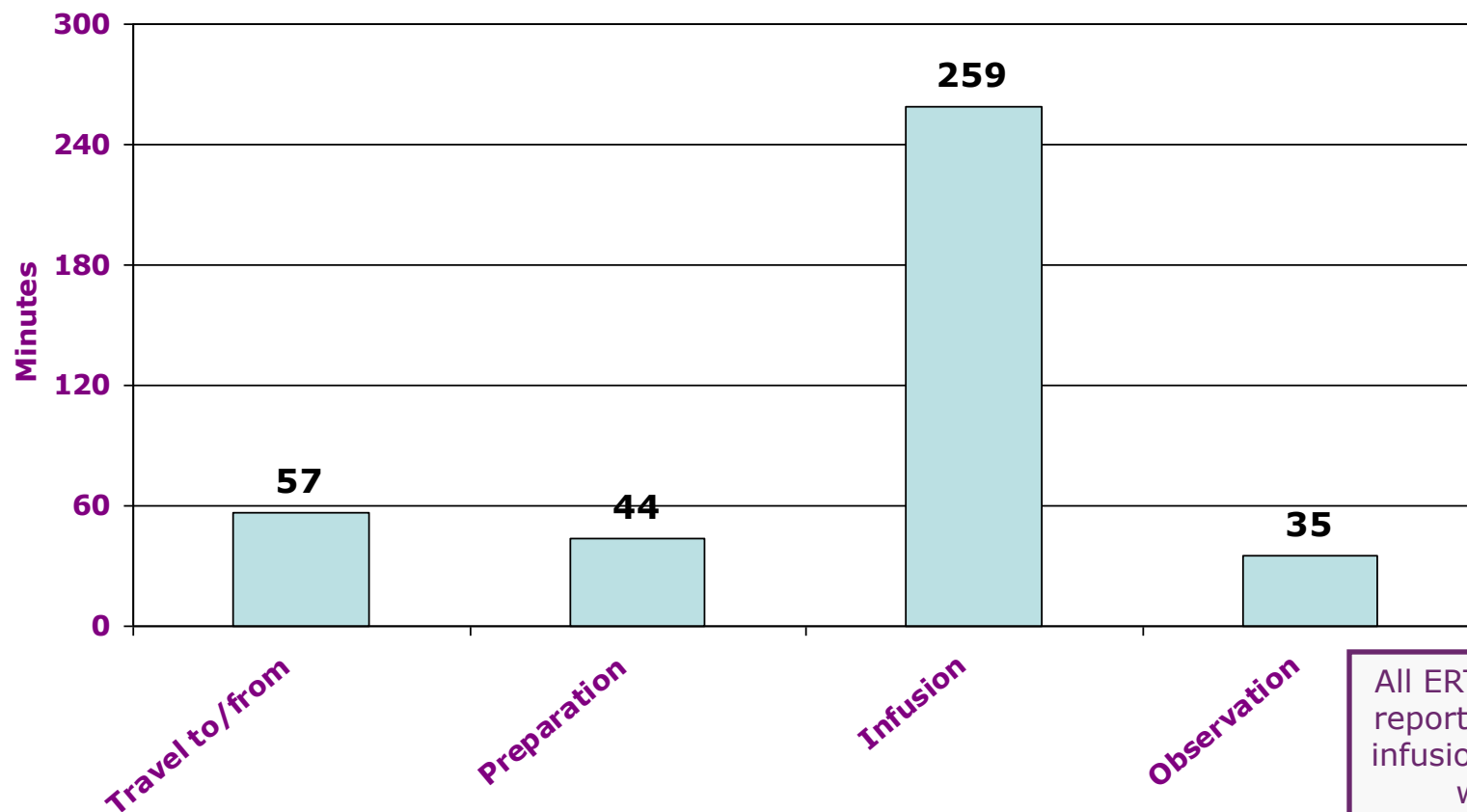
Highest home infusion among 6<18 year olds, Female



**ERT TIME
COMMITMENT**

On average, patients devote more than 6 ½ hours to each weekly infusion (including 4 ½ hours of infusion time)

**Time of Steps in Infusion Process
(minutes)**



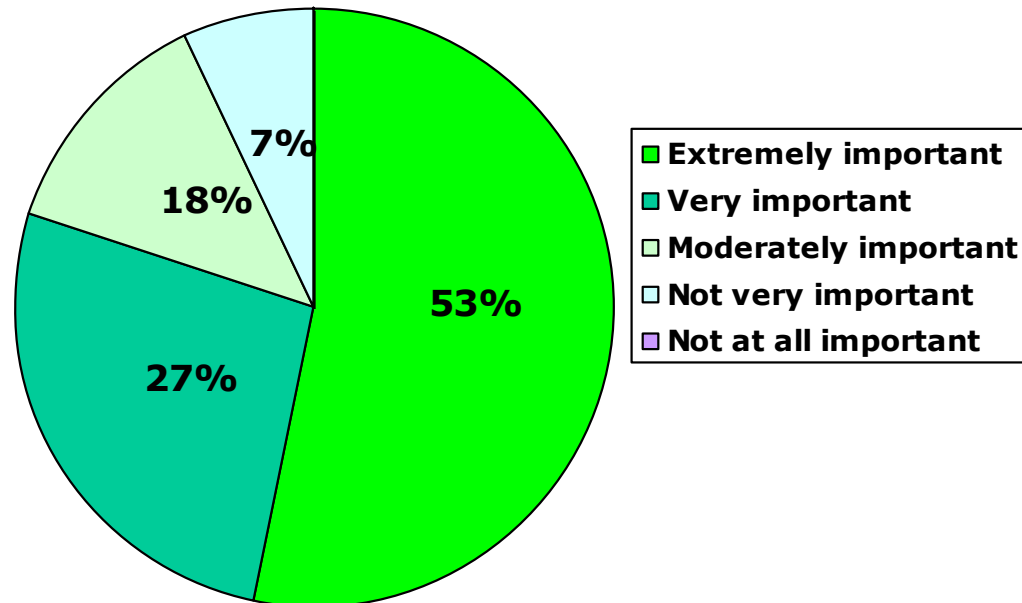
Q30. Please estimate how much time, in minutes, it takes/took for the following steps of the infusion process. (N=29)

ERT COMPLIANCE

Compliance is nearly total, and $\frac{3}{4}$ say it is very/extremely important not to miss any infusions

Importance of not missing any infusions

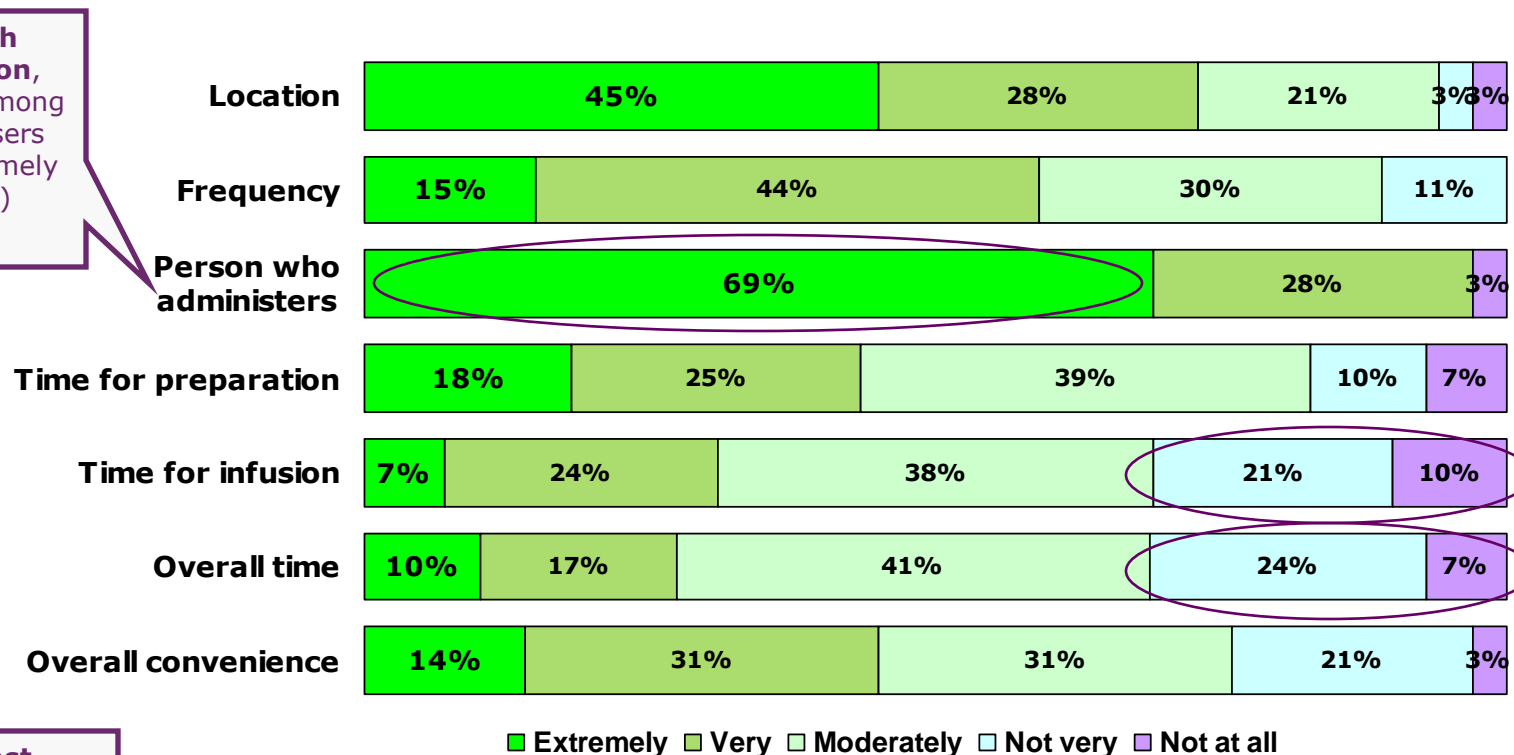
Only 1 respondent reports missing four or more consecutive infusions; the reason given was hospitalization



Q20. Have/had you ever missed 4 or more consecutive infusions? (N=29) Q21. Which of the following items, if any, describe the main reasons you missed or stopped ERT? (N=1) Q22. How important would you say it is to receive ERT without missing any infusions? (N=30)

There is general satisfaction with administration of Enzyme Replacement Therapy; however, 1 in 3 patients report dissatisfaction with overall time commitment and infusion time (there is less dissatisfaction with infusion frequency)

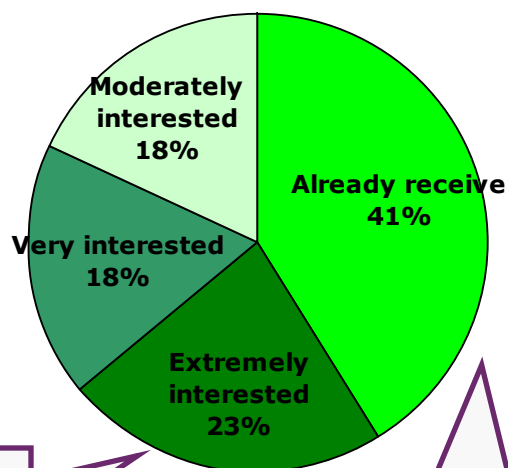
Satisfaction with Administration of ERT



The **most discontent on time** is among age 18+, Male

Home infusion has been embraced by many MPS I patients, and many others express high interest; self-infusion also draws some interest

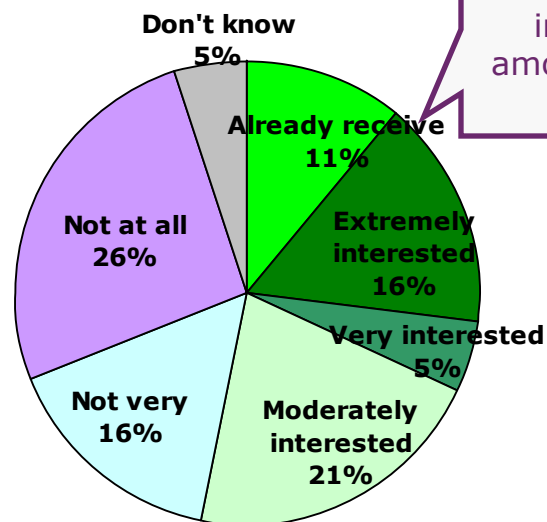
Home infusion



The most interest is among Severe, Male, age <6

The most current usage is among Female, age 6<18

Self-infusion



The most current use and additional interest is among Severe, age <6

Q32. How interested would you be in each of the following types of ERT administration and infusion methods? **Home infusion:** A nurse or clinician comes to your home to prepare the solution and administer the infusion. **Self infusion:** You/Parent/Guardian . . . administer the infusion on your own. (N=22 currently on ERT)

The main reasons given for not receiving Enzyme Replacement Therapy are lack of availability at the time & appropriateness of transplantation

Reasons for NOT receiving ERT

Volunteered (multiple response accepted)

Opted for transplantation

Not available to us/at time of transplant/not available when diagnosed/ was not approved yet	38%
Had transplant/BMT/stem cell transplant/transplant was successful	31%
Enzyme level sufficient after transplant/no need for	10%
Chose transplant/more permanent	7%
No wait for transplant/would have delayed transplant/went into transplant as soon as donor found	7%
Afraid of transplant rejection if ERT given prior to	3%
Transplant worked well for first child	3%

Physician discouraged ERT

Genetic doctor claimed only for more serious patients/did not qualify	7%
Doctors felt no benefit after transplant/we felt would be redundant	7%

Cost concerns

Too expensive	3%
Concerns over insurance coverage	3%

Other

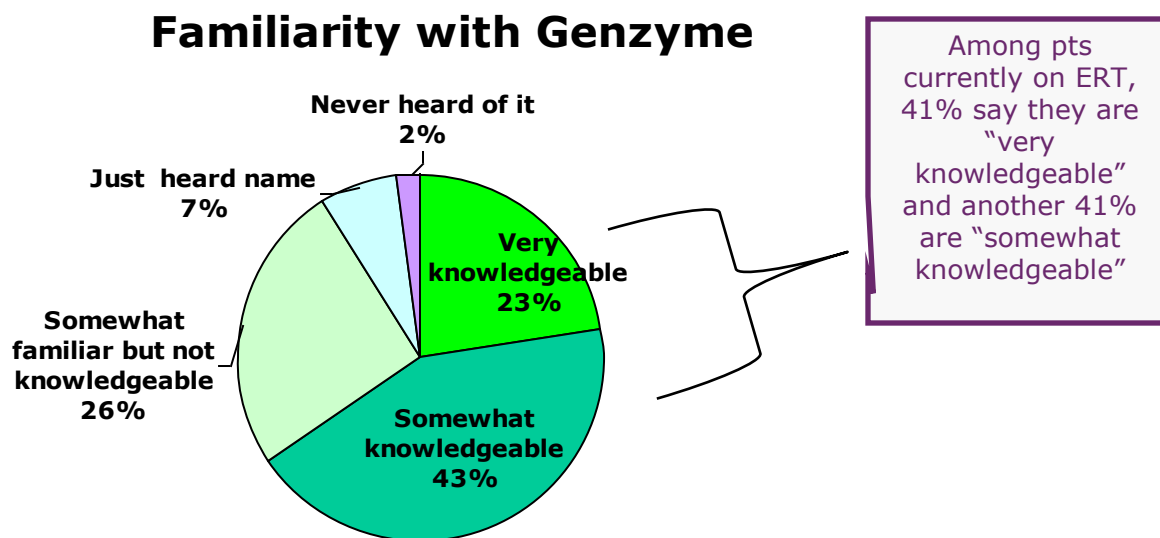
Just diagnosed/will start treatment soon	3%
All other	3%

No comment	10%
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Patient familiarity with Genzyme is quite high



Q34. How familiar would you say you are with **Genzyme**, the manufacturer of Aldurazyme enzyme replacement therapy for MPS I disease? (N=61)

Patients express very positive attitudes toward Genzyme; appreciation focuses on the responsiveness of Genzyme support services; also on the comprehensiveness/ relevance of disease/treatment information

How Has Genzyme Helped?

Volunteered (multiple response accepted)

Support

Help with insurance coverage/insurance problems	16%
Help us find local doctor/hospital for treatment/setting up infusion sites	13%
<u>Excellent</u> customer service/support/was there before, thru infusions/ to answer questions	13%
Conferences/helped us attend/sponsored me	7%
Case Management Specialist/available for consultation/have <u>good relationship</u>	5%
Funding research/for new treatments/current therapies/FDA approval	3%
Getting hospital to add drug for treatment/training for hospital to do ERT	3%
Help with fundraising/ annual meetings	5%
Provided funding for website	2%
Drug has reduced several of my symptoms	2%

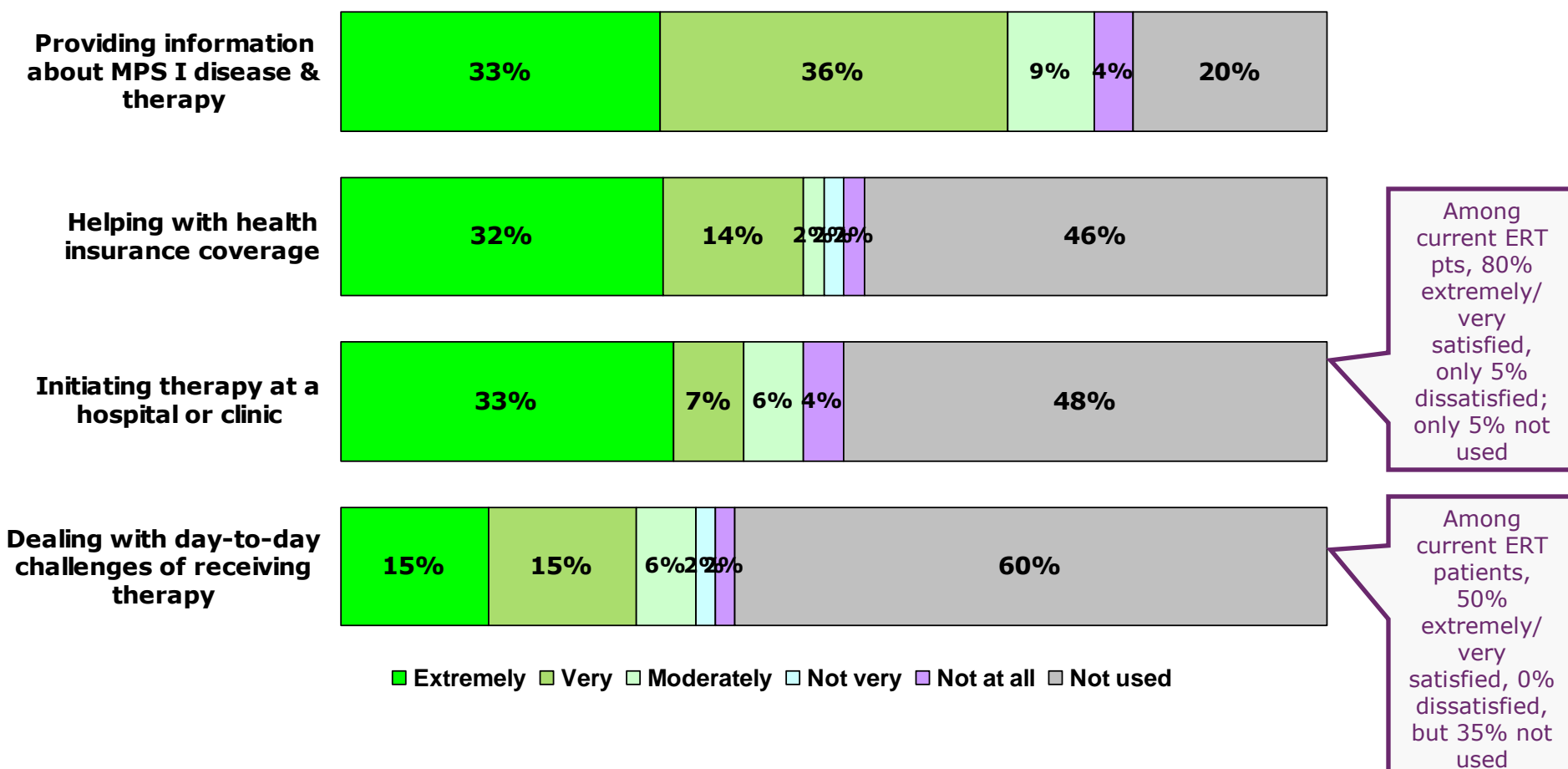
Information

Mailings/resources/family resource guide/information sent out to families	13%
Knowledge/to hospital staff/myself on information regarding disease/ to help answer my questions	7%
Website/resources online/ <u>very informative</u>	5%
Notebook/Hurler/binder	2%
Given materials/options on treatments	2%
Provided materials about MPS I (unspecified)	2%

None/N/A/No comment **36%**

Patients indicate widespread use and satisfaction with Genzyme information; satisfaction is also very high on other support

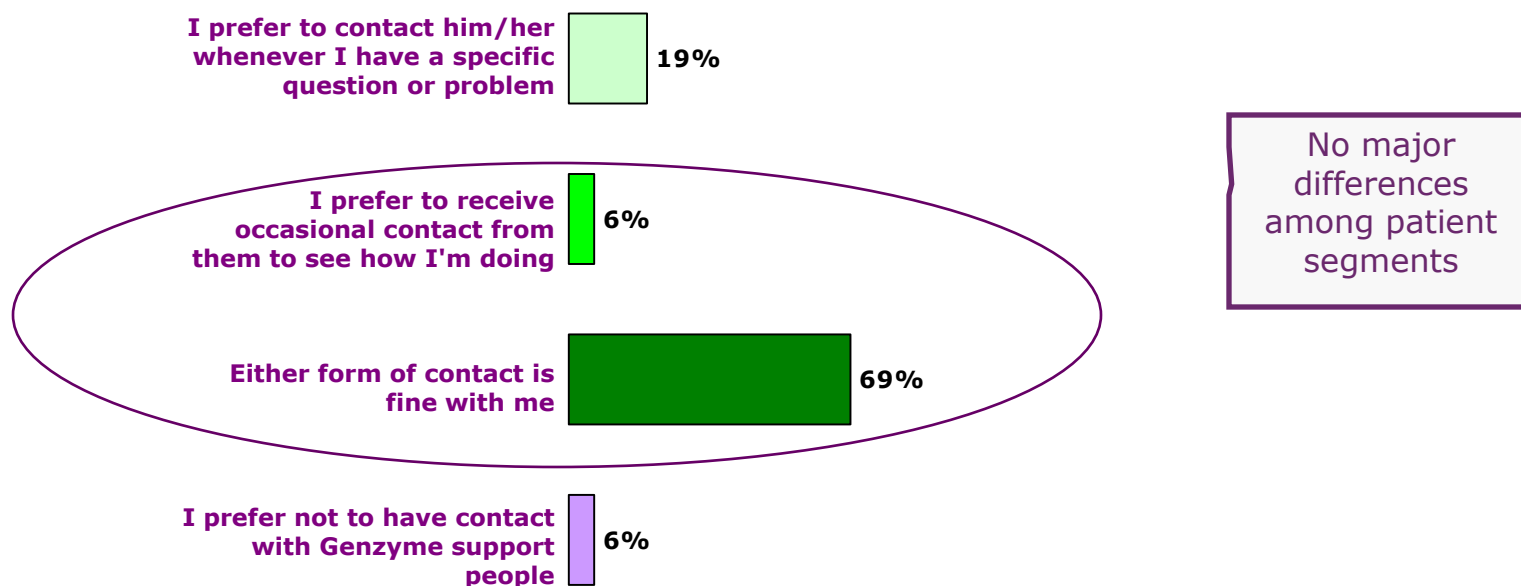
Usage & Satisfaction with Genzyme Support Services



Q36. Please indicate your level of satisfaction with each of the following Genzyme support services. (N=56)

$\frac{3}{4}$ of patients are open to receiving proactive communications from Genzyme

Contact Preference with Genzyme Support People



Additional information-sharing tops the list of suggestions for Genzyme service improvement, including holding more informational meetings with patients

How Can Genzyme Better Serve?

Volunteered (multiple response accepted)

Information

Keep us informed/up to date on treatments/studies/medical trials/ how they are going/next phases	7%
Organize meetings/share information with other families/share experiences/ problems with receiving ERT	5%
Want more information (unspecified)	3%
Keep website updated	3%

Support

Better contact/support/follow up from reps/case manager	3%
Help with fundraising	3%
Keep funding MPS Society	3%
Help with insurance coverage	3%

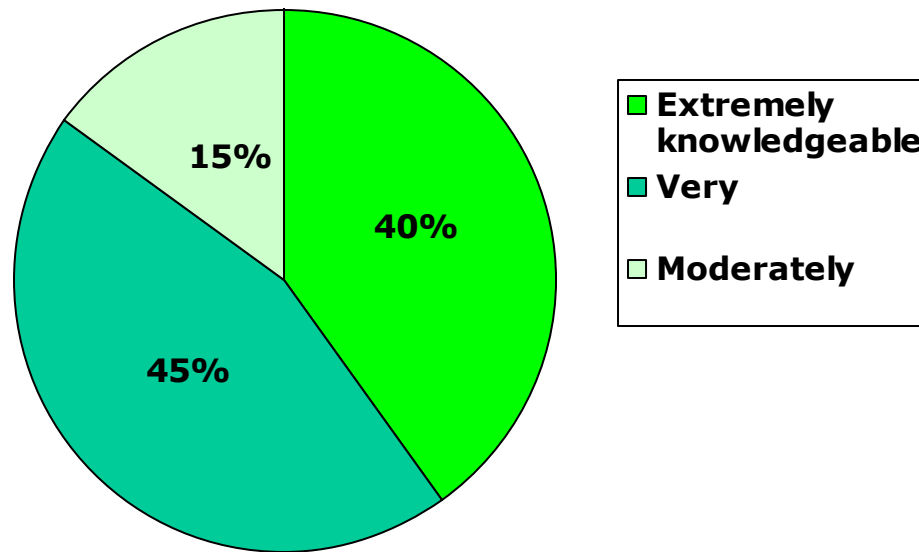
Other

Lower cost	3%
Improvements in treatments/ shorten times of infusions	3%
Keep up the good work	3%

None/do not need/can't think of anything/no comment	75%
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Most patients say they are knowledgeable about MPS I and treatment

Patient Knowledge of Disease & Treatment



No major differences among patient segments

***Disease* information needs appear to be met; unmet information needs focus on what to expect from *therapy* plus recent advances in therapy**

Additional Information Needs

Volunteered (multiple response accepted)

Effectiveness of therapy, outcomes

Info/statistics regarding children with disease/had transplants/ <u>what to expect/</u> post transplant studies	10%
ERT information/what it corrects/stops/helps with	7%
Concerns of skeletal/dental issues/post transplant	5%
Collect/share data/detailed reports/database on <u>patient outcomes/</u> after treatment	5%
Questions on side effects cause by treatments/medicine/weight gain/ loss of appetite	5%
ERT after BMT or not?	3%
Information on MPS I <u>long term effects/expectancies</u>	2%
More information on BMT/bone marrow transplant	2%
What needs to be done after treatment/tests <u>run/how often to see doctor</u> etc.	2%

Advances in therapy

On new advances being made/for after transplant/new treatments/hoping for cure	8%
Information on clinical trial reports	3%
Intrathecal infusion of Aldurazyme	2%
Would like to receive current information/not much available to me	2%

Cost of therapy

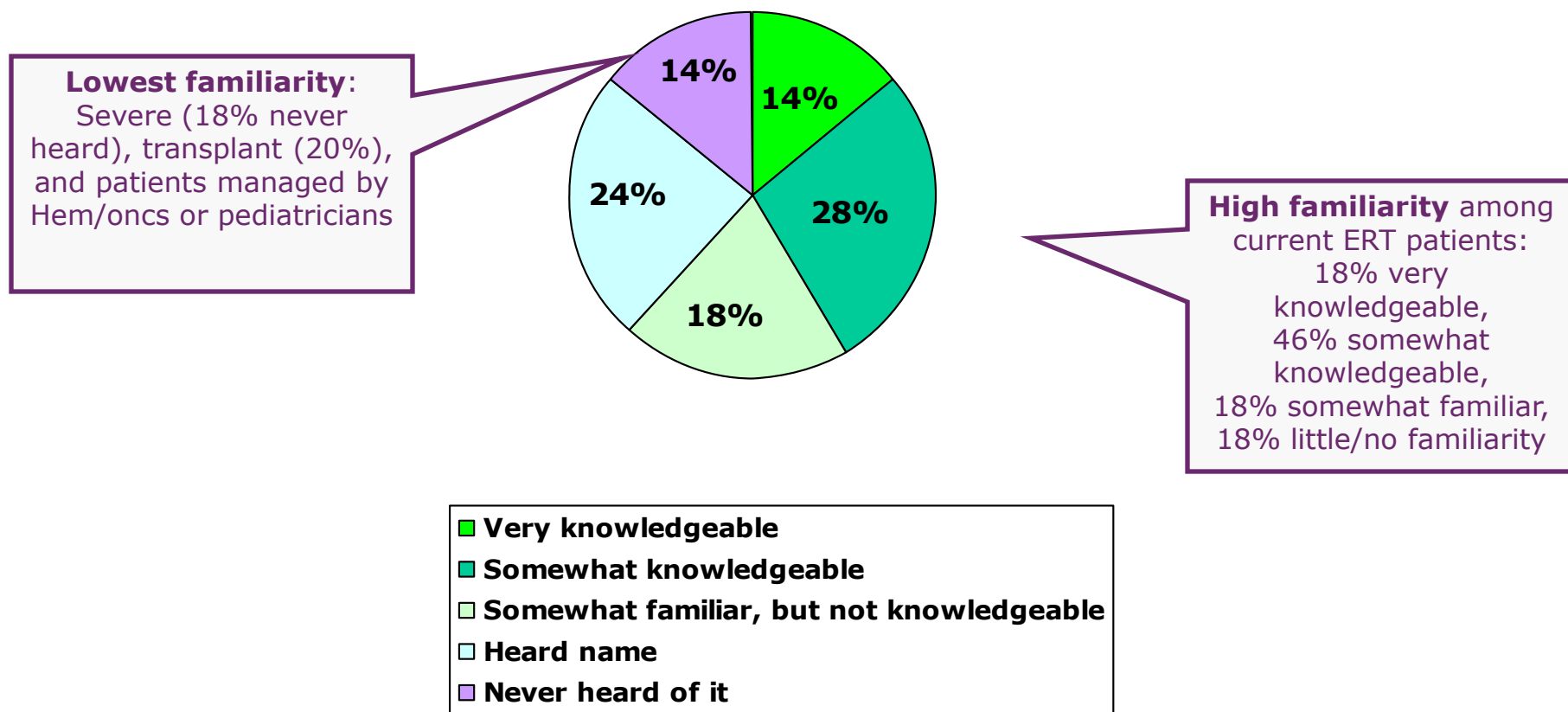
Financial aid	2%
Cost of ERT/if problems arise with insurance coverage	2%

None, have everything, no comment	49%
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Q44. What information—about MPS I disease, its treatments, and/or support services—would you like to receive that you don't already have? (N=61)

Although a significant number of patients know about the Registry, others need to learn about it

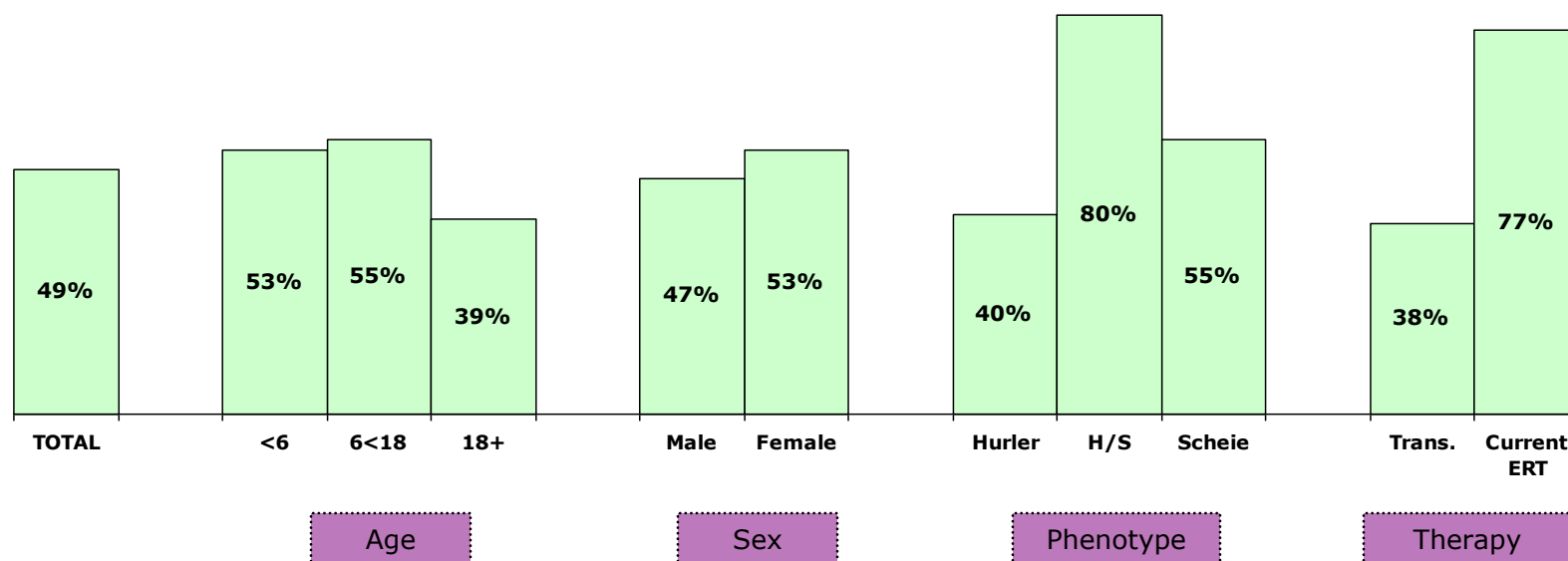
Familiarity with MPS I Registry



Q39. How familiar are you with the **MPS I Registry**, a clinical database of confidential patient care information that helps physicians monitor a patient's progress, determine which treatments lead to better patient outcomes, and provides a global resource for MPS I treatment advancement? (N=59)

½ of MPS I pts say they are enrolled in the Registry; among those not enrolled (or not sure) about ½ express interest in registering

Patients Reporting They are Enrolled in the MPS I Registry



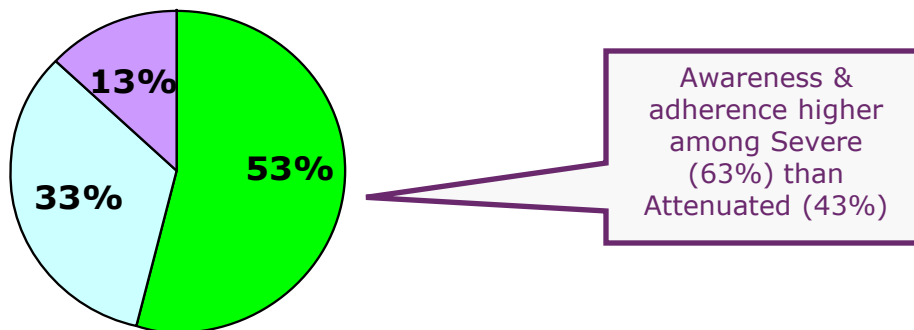
18% say they are **not enrolled**, 33% don't know

Among these, 55% are **interested in registering**, only 3% are not interested, 42% unsure

Q40. To the best of your knowledge, are you registered with the MPS I Registry? (N=61) (If “no” or “don’t know”) Are you interested in registering with the MPS I Registry? (N=31)

Just ½ of MPS I pts enrolled in the Registry adhere to the monitoring schedule; many say they don't receive the schedule

Awareness & Adherence to Registry's Minimum Schedule of Assessments
(among registered in MPS I Registry)

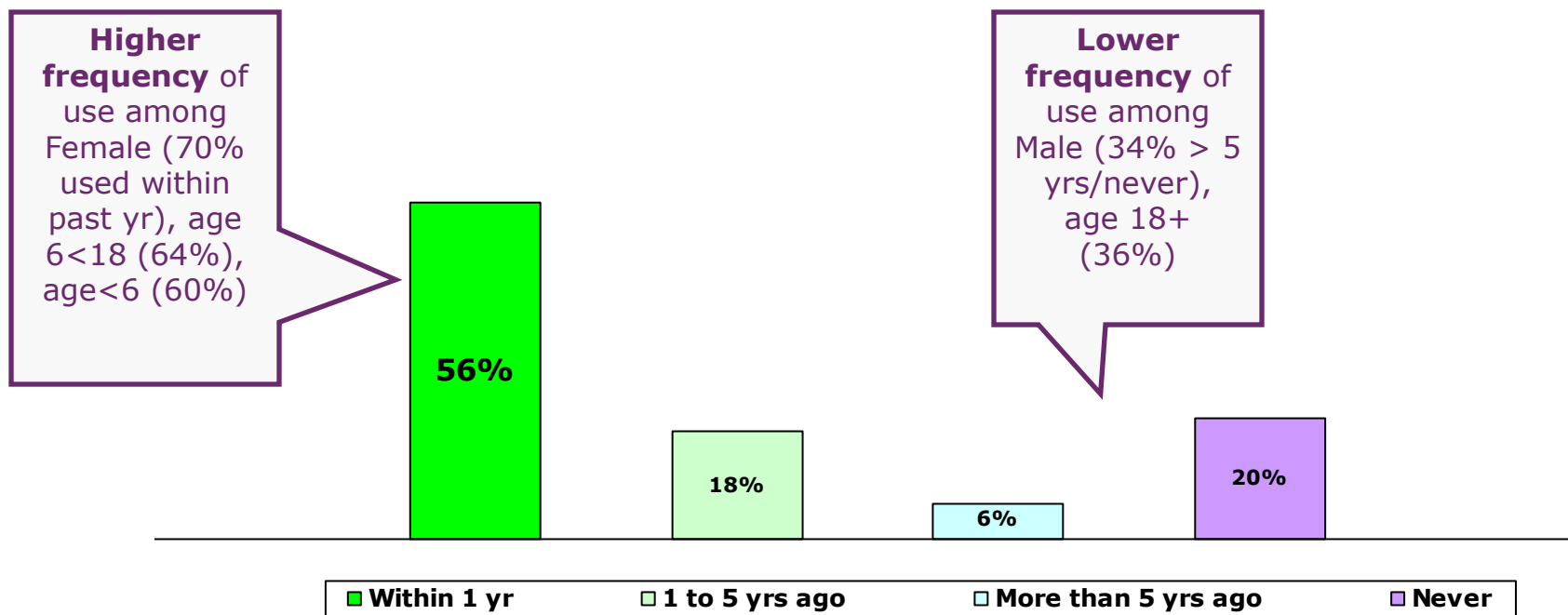


- **Aware, and adhere to schedule**
- **Aware, but don't receive/don't know of schedule**
- **Not aware**

Q41. The MPS I Registry's recommends a "Minimum Schedule of Assessments for Monitoring Patients with MPS I", that is, periodic exams to monitor your health on a variety of symptoms. What is your level of awareness/use of these symptoms health assessments? (N=30)

Slightly more than 1/2 of members have used MPS Society services within the past year; usage appears less frequent among Attenuated and adult patients

When Last Used Services of National MPS Society



95% of respondents indicate they are members

Only 1 respondent is not a member because "don't think they can help anymore"

Q45. Are you a member of the **National MPS Society**? (N=61) What are the reasons why you are **not** a member of the National MPS Society? (N=2) Q47. When, if ever, did you last use the services of the National MPS Society? (N=55)

Members most often think of the Society's conferences, information resources, and family & physician referrals

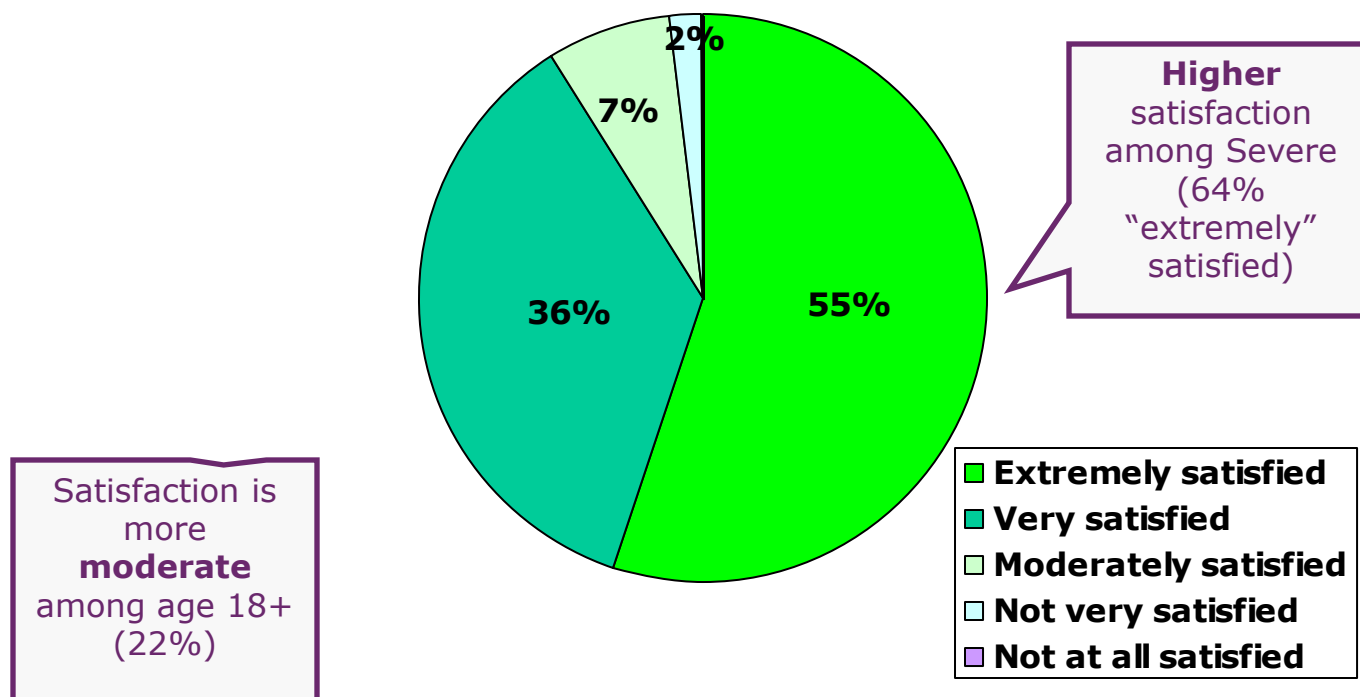
Services Received from the National MPS Society

Volunteered (multiple response accepted)

Conferences/seminars/scholarship to/conference assistance	31%
Information/educational material/booklets/handbook/ Courage Magazine/newsletter	21%
Information about disease	11%
Website resources	8%
Information on ERT	2%
Information package for our pediatrician/doctor	2%
Mailings (unspecified)	2%
Updates (unspecified)	2%
Support/contact information/advice	8%
Family references/family contacts/info on friends	5%
Referral to BMT	2%
Financial help with membership	3%
Fundraising	3%
Many/all that is needed (unspecified)	7%
No comment	33%

90% of active members are extremely/very satisfied with National MPS Society services

Satisfaction with National MPS Society



Q49. What is your overall satisfaction with the services provided by the National MPS Society? (N=42 pts who have used society services)

There were few recommendations for improvements in Society services; most comments focus on updated info

How can the National MPS Society Better Serve?

Volunteered (multiple response accepted)

Happy with MPS Society/ is wonderful/do a fantastic job/keep us informed well	10%
Updated information	
Keep website updated/more often	3%
Keep mailings updated	2%
Keep us updated on outcome of treatments/patient updates/how are they now?	2%
More transplant information/less ERT	2%
Parent/Child/Family	
Parent forum online/connect with other parents/families	2%
Better family support	2%
More resources for children	2%
Other	
Conference assistance	2%
List of doctors/familiar with MPS/by state	2%
Don't know, no comment	49%

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SUMMARY

Patients' assessment of their health

- ❑ **A large majority of MPS I patients give positive assessments to their overall physical and emotional health**
- ❑ **Highest ratings are on quality of life, with 2/3 rating it “excellent” or “very good”**
- ❑ **A significant minority of patients give negative ratings on physical symptoms, especially musculoskeletal (59% fair/poor) and vision (46%)**
 - **The most problems on these measures are reported for Severe patients, patients age 6<18, and Attenuated patients**

SUMMARY

Patient satisfaction with transplantation

- ❑ **Many transplantation recipients say they experienced complications (72%) and repeat procedures (22%)**
- ❑ **However, most patients report high satisfaction with their transplantation, with many saying it “far exceeded” their expectations**
- ❑ **Fully 84% of transplantation recipients say transplantation provided “a lot” of improvement to their quality of life**
- ❑ **Majorities of transplantation recipients report “a lot” of improvement on respiratory and gastrointestinal symptoms, and most report at least “some” improvement on other symptoms**
- ❑ **The lowest improvement scores are on vision and musculoskeletal where 26% and 22% report little/no improvement**
 - **The younger that patients receive a transplantation the better their reported outcome on musculoskeletal issues**

SUMMARY

Patient satisfaction with Enzyme Replacement Therapy

- ❑ **Enzyme Replacement Therapy satisfaction varies widely; it has met or exceeded expectations for 46% of patients, fallen short for 38%**
 - **The most satisfaction is among Severe patients, age < 6 and former ERT patients (i.e., those who took ERT in combination with transplantation), suggesting that early treatment leads to the best outcomes**
 - **The most dissatisfaction is among Attenuated patients, females, and age 6<18**
- ❑ **The most improvement from Enzyme Replacement Therapy is reported on quality of life, abdominal, respiratory & cardiac parameters; least improvement reported on mental development & vision**
 - **The longer that patients receive Enzyme Replacement Therapy, the better their reported outcomes, especially dealing respiratory symptoms, plus musculoskeletal and vision**

SUMMARY

Compliance, Convenience

- ❑ **Compliance is near total, and most patients say it is very important not to miss any infusions**
- ❑ **There is general satisfaction with administration of Enzyme Replacement Therapy**
 - **Very high satisfaction with home care nurses**
 - **However, 1 in 3 respondents report dissatisfaction with infusion time and overall time commitment (there is less dissatisfaction voiced regarding frequency of infusion)**
- ❑ **Fully 41% of current Enzyme Replacement Therapy patients report they receive home infusion; all the rest are at least somewhat interested including 23% “extremely interested” in adopting home infusion**

SUMMARY

Support, Information Needs

- ☐ **Nine out of 10 active members say they are extremely/very satisfied with the National MPS Society**
- ☐ **Patients express very positive attitudes toward Genzyme**
 - **Appreciation focuses on the responsiveness of Genzyme support (“excellent customer service”)**
 - **Also on the comprehensiveness/relevance of disease/treatment information (“very informative”)**
- ☐ **The top request for more information regards “what to expect” of treatment**
 - **Patients welcome detailed, statistical information, “statistics regarding children with disease,” “Collect/share data/detailed reports/database on patient outcomes/after,” “What needs to be done after treatment/tests run/how often to see doctor ”**
- ☐ **The MPS I Registry can be deployed more to help treat & inform patients**
 - **½ of patients say they are registered with the Registry, and of these, about ½ are aware and adhere to the minimum schedule of patient monitoring**

