

FAMILY VOICE → TREATMENT DEVELOPMENT

Defining Success in DESSH

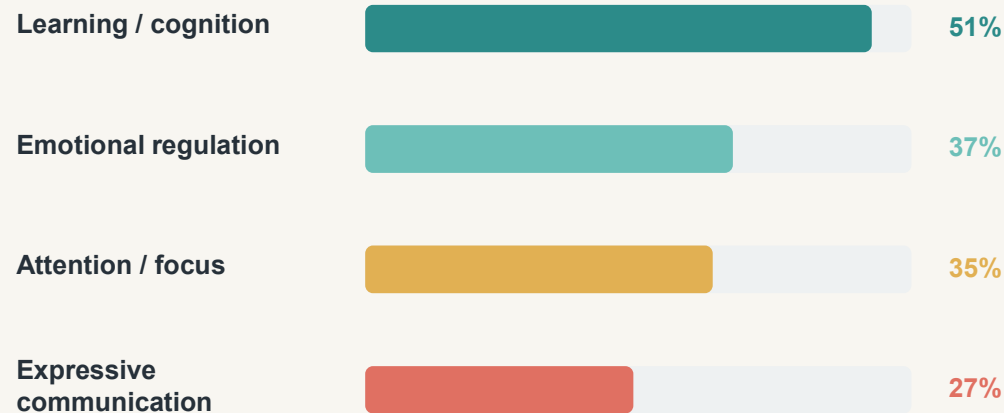
What would make a future treatment meaningful—and worthwhile—to people with DESSH and their families?

2026 DESSH Family Conference

WHAT FAMILIES PRIORITIZE

There is no single universal treatment target

SELECTED IN TOP 3



WHEN FORCED TO CHOOSE ONE



The top answers are close—and priorities vary across the DESSH community.

SURVEY TAKEAWAY

Researchers may need to plan for more than one meaningful treatment outcome.

WHAT FAMILIES ULTIMATELY WANT TREATMENT TO MAKE POSSIBLE

Symptoms are not the end goal

56%

Greater
independence

42%

Improved ability to
learn

37%

Better overall
quality of life

35%

Improved emotional
well-being

DESSH feature



Observable functional
change



Real-life outcome



Meaningful
benefit

Independence may not be the biological target, but it is often the downstream goal.

A STRONG SIGNAL FROM FAMILIES

Treatment does not have to change everything.

91%

would definitely or probably consider a treatment successful if it improved **ONLY** their highest-priority area.

44%

Even a small improvement could be meaningful

26%

Need a moderate improvement

14%

Need a large improvement

Meaningful doesn't necessarily mean dramatic.

TREATMENT TRADEOFFS

Benefit has to justify risk

42%

Some risk for a
meaningful improvement

7%

Substantial risk for a
major improvement

28%

Unsure

Families appeared more open to treatment burden than to substantial treatment risk.

74%

Daily oral

60%

Specialty
visits

60%

One-time
hospital
procedure

53%

Monthly
injection

14%

Lumbar
puncture

INTERPRET CAUTIOUSLY

Risk was not tied to a specific adverse event or probability; 28% were unsure.

Now: turn a priority into meaningful change

1

WHAT MATTERS?

Choose the ONE thing you would most want a treatment to improve.

2

WHAT CHANGES?

What specific change would you notice in everyday life?

3

WHAT BECOMES POSSIBLE?

What could the person with DESSH do that is difficult today?

Be concrete. There are no wrong priorities—and we do not need consensus.

DISCUSSION

When is the benefit worth the risk?

Imagine a treatment has a reasonable chance of producing the meaningful change you just described.

Would that benefit be worth...

- a daily medication?
- a monthly injection?
- regular monitoring?
- a small but serious medical risk?

A treatment decision is not one question.

What benefit?

How much?

How likely?

What burden?

What risk?

The goal is not to establish a risk threshold by show of hands.
It is to understand the tradeoff.

ONE SENTENCE

Define success for your family.

A successful DESSH treatment would...

There is no single right answer.

**Meaningful change
is real-life change.**

**“Did that change make
life meaningfully better
for the person with
DESSH?”**

**A change that looks small on a clinical scale can have a very large impact
on everyday life.**

From changing WAC biology → to changing what life makes possible.