SMA Conjoint MR – Demand Estimation

HCP and Patient Quantitative Findings

November 2024

PREPARED BY:
COGNITIVE CONSULTING

CONTENTS

Evrysdi risdiplam

OBJECTIVES & METHODOLOGY

PROJECT OBJECTIVE: To understand opportunities and measure the future uptake of Evrysdi with future competitors for Genentech's long-term forecast within the SMA treatment market.

1. Identify Evrysdi Opportunity Within the SMA Market
2. Evaluate the Current SMA Treatment Market Share of Evrysdi and Its Competitors
3. Measure the Base Case and Future Uptake of Evrysdi with and without the Availability of the Evrysdi Tablet
4. Assess Current Competitor Product Perceptions and Interest in Competitor Products in Development

METHODOLOGY

Quantitative Online Survey with Hybrid Conjoint

- Conducted from 9/20/2024 – 10/22/2024
- Survey length: 45 min for HCPs and 30 min for Patients/Caregivers

QUANT SAMPLE

n=100 Patients

- ***n=65*** *Adult Patients*
- ***n=35*** *Pediatric Caregivers*

n=100 HCPs

- ***n=57*** *Adult Neurologists*
- ***n=43*** *Pediatric Neurologists*

WHO WE TALKED TO: HCPs

Sample

n=100 Neurologists

n=57 *Adult Neuros*

n=43 *Pediatric Neuros*

of SMA patients/year

Total Patient Representation:

3,884 Patients

Average:

38.8 Patients

Average currently treated:

20.4 *patients*

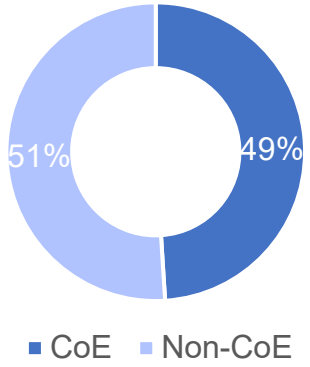
Average previously treated:

10.3 *patients*

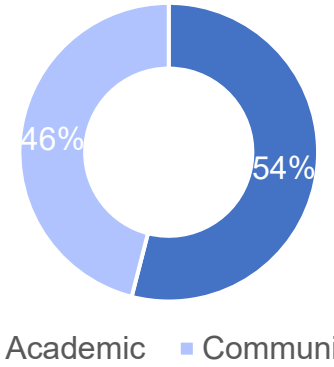
Average never treated:

8.0 *patients*

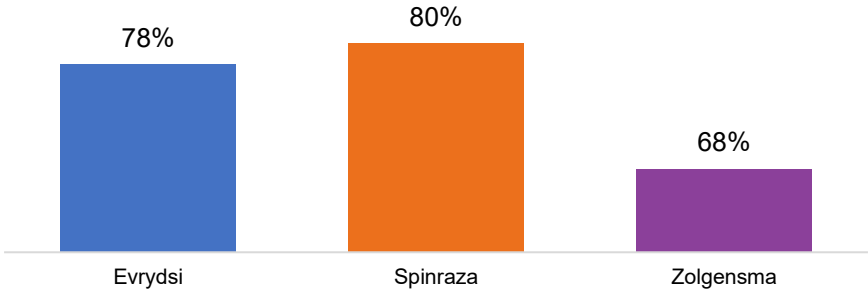
Center of Excellence



Treatment Setting



Prescribe SMA DMT



Average Number of Patients on DMT

WHO WE TALKED TO: PATIENTS

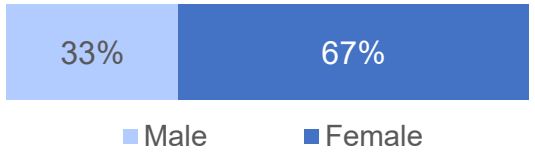
Sample

n=100 Patients

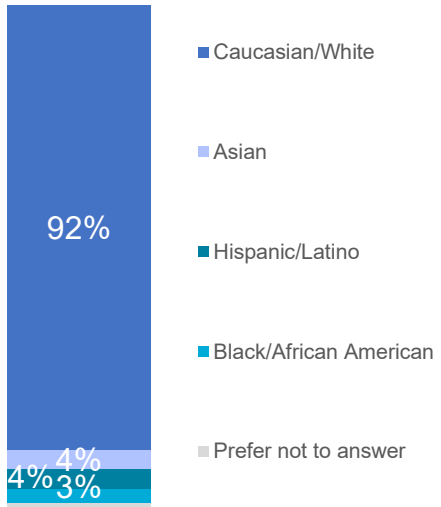
n=65 *Adult Patients*

n=35 *Pediatric Caregivers*

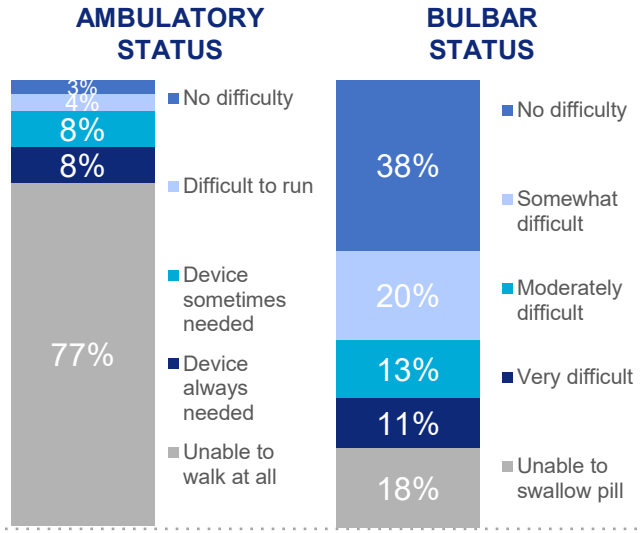
Patient Gender



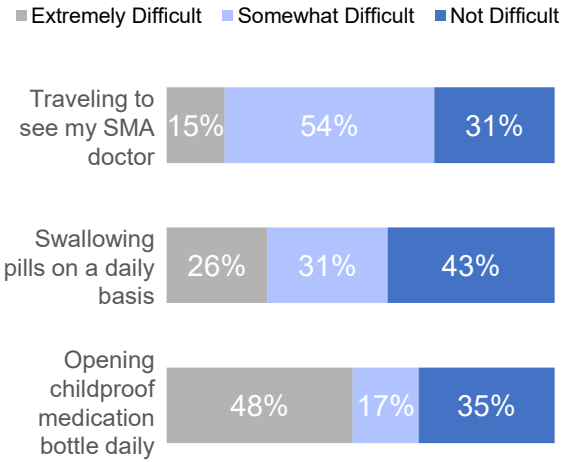
Patient Ethnicity



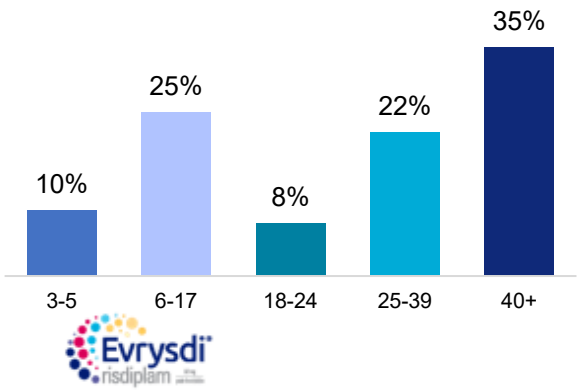
Ability Status



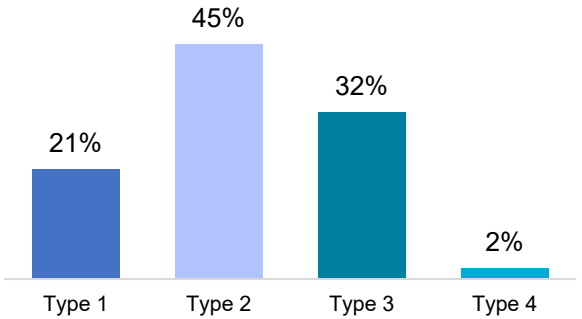
Difficulty With…



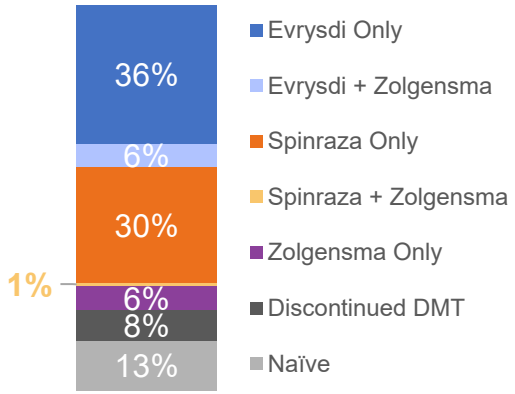
Age



SMA Type



Current Treatment



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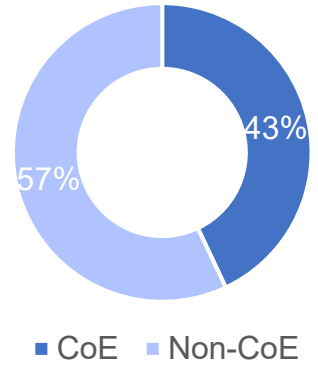


KEY FINDINGS

Evrysdi® risdiplam 60 mg powder for oral solution

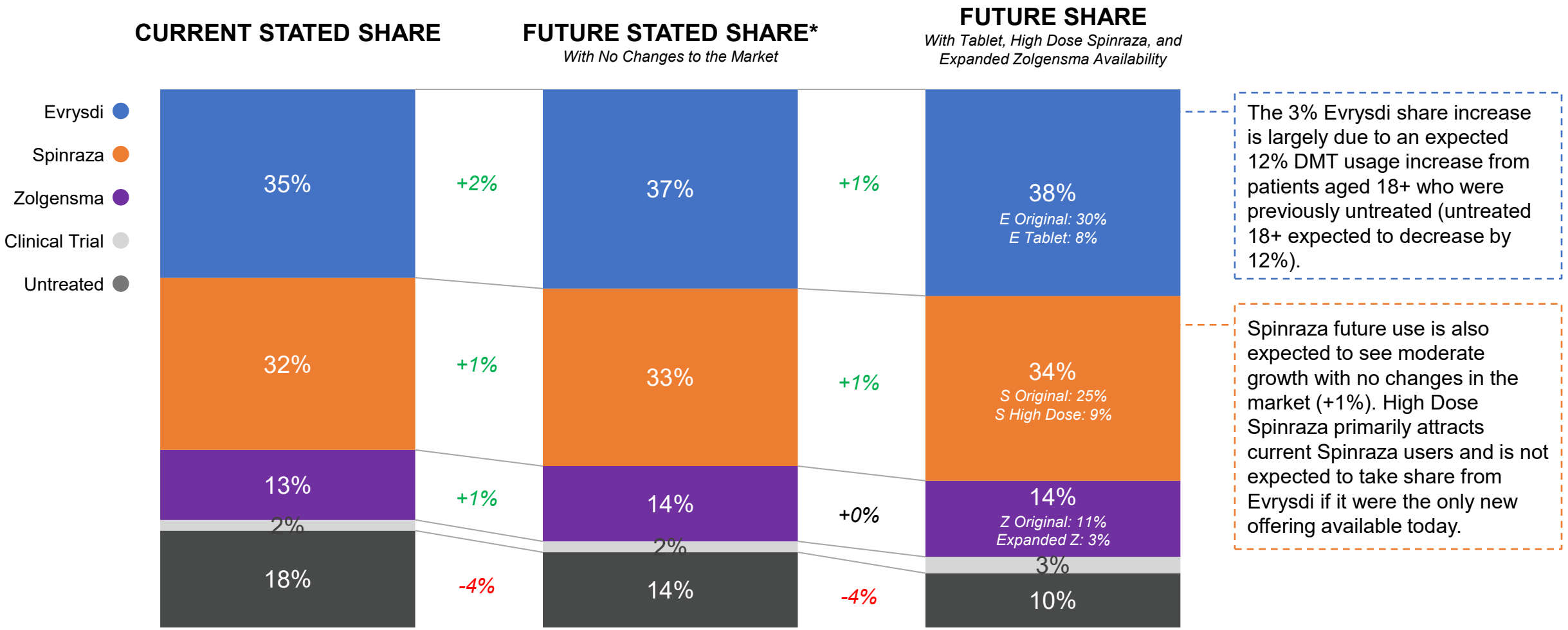
KEY INSIGHTS

PATIENT | HCP

OBJECTIVE	FINDINGS
Identify Evrysdi Opportunity Within the SMA Market.	• Evrysdi's current impression in the market among both patients and HCPs remains strong and allows for the opportunity for growth through switches and new patients with and without the availability of the Evrysdi Tablet. • Adults SMA patients (18+) have a strong influence on the SMA market share, and many are interested in starting a new treatment in the next 12 months. • The Evrysdi Tablet offers a benefit that both current and non-Evrysdi users have interest in, allowing for both expansion and conversion. • While DEVOTE and STEER offer benefits as well, growth is expected to be limited to primarily conversion of current Spinraza patients and expansion from untreated patients and is unlikely to motivate current Evrysdi patients to switch.
Evaluate the Current SMA Treatment Market Share of Evrysdi and Its Competitors.	• Evrysdi currently holds the strongest share in the SMA treatment market (35%) compared to its competitors, Spinraza (32%), and Zolgensma (13%). • Adults (18+) make up a majority of the SMA patient population and have the largest impact on total share. Evrysdi (34%) and Spinraza (33%) hold similar shares of the adult patient population. • 18% of SMA patients (30% of adult SMA patients) are currently untreated.
Measure the Base Case and Future Uptake of Evrysdi with and without the Availability of the Evrysdi Tablet.	• Patients and HCPs expect an increase in future SMA treatment use among currently untreated patients, without any new products introduced in the market. Evrysdi (+2%), Spinraza (+1%), and Zolgensma (+1%) are each expected to see usage growth in the next 12 months. • With the availability of the Evrysdi Tablet, High Dose Spinraza, and the Expanded Indication of Zolgensma, Evrysdi's share is expected to grow an additional 1% with expansion primarily influenced by currently untreated adults seeking treatment.
Assess Current Competitor Product Perceptions and Interest in Competitor Products in Development.	• Patient awareness of High Dose Spinraza is high (71%) compared to the Evrysdi Tablet (34%), however, the impression of the Tablet (73%) outperforms other products (56% DEVOTE impression, 45% STEER impression). • Interest and likelihood to talk to an HCP about all three products are comparable, however HCPs indicate an expectation of Evrysdi share growth through the introduction of the Tablet. • The introduction of High Dose Spinraza and the expanded indication of Zolgensma are expected to have minimal impact on Evrysdi share, with both Spinraza and Zolgensma share growth stemming from untreated patients, current Spinraza users, and pediatric patients.

SMA TREATMENT SHARE

Without any change to the current market, Evrysdi is expected to see a slight increase in market share based on both HCP and Patient sentiments. With the introduction of the Evrysdi Tablet, High Dose Spinraza, and the expanded indication for Zolgensma, Evrysdi is expected to capture additional share primarily from SMA patients who are currently untreated.

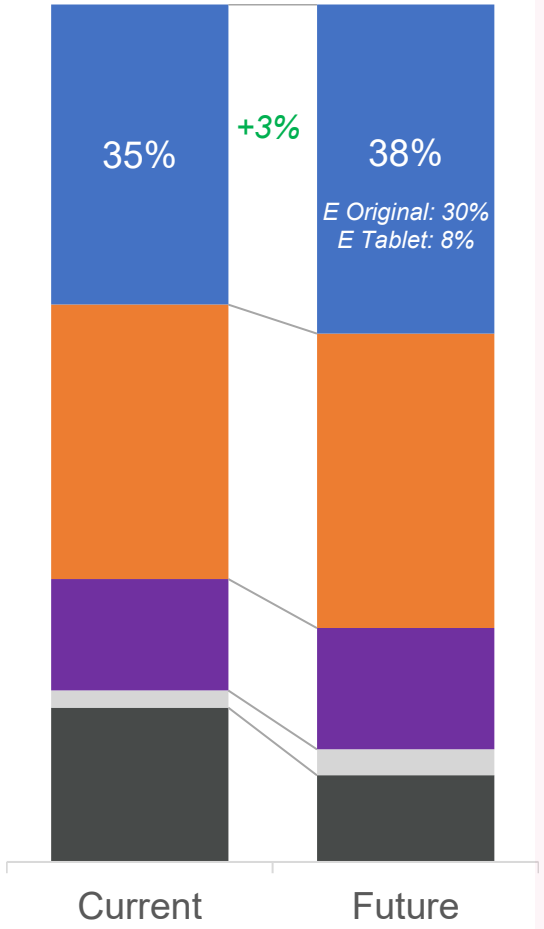


*Note: Future stated shares with no changes to the market are calculated based on a combination of HCP future expectations with no changes in the market (B8-B10) and patient stated future treatment expectations (B5-B6, B9-B10).

HCP

HCP SHARES

Among Total HCPs (n=100)



HCP INSIGHTS

HCP share data indicates a 3% growth for Evrysdi with the availability of the Evrysdi Tablet.

Of the 38% share…
- 79% are expected to be on the original formulation
- 21% are expected to start on the Evrysdi Tablet formulation

Of the 3% share growth…
- 2% is expected with NO change in the market
- 1% is due to the Tablet introduction

IMPLICATION: Non-Evrysdi users starting on Evrysdi make up the 3% growth, with over half expecting to start on the original formulation.

QUESTION: Why is the Evrysdi Tablet share significantly lower than the original formulation share? Patient insights are used to triangulate these findings.

PATIENT

	EVRYSDI USERS	NON-EVRYSDI USERS
Total	n=42	n=58
Eligible for Tablet	79% *(n=33)*	81% *(n=48)*
No Issues Swallowing Pills *Among Eligible*	30% *(n=10)*	56% *(n=27)*
No NG Tube Use *Among Eligible*	70% *(n=23)*	92% *(n=44)*
Interest in Tablet *Rated 5-7, Among Eligible*	70% *(n=23)*	46% *(n=22)*

Patients who are **most likely** to convert to Tablet are:
- Eligible for tablet
- Have no issues swallowing pills
- Do not use an NG tube
- Very interested in the Tablet (rated 5-7)

21% of current Evrysdi users fall into this category (n=9)

31% of non-Evrysdi users fall into this category (n=18)

Evrysdi user patient insights applied to HCP share proportions:
- Future Evrysdi share that consists of current Evrysdi patients = 35%
- 21% of that 35% = **7%** expected to convert to the TABLET

Non-Evrysdi user patient insights applied to HCP share proportions:
- Future Evrysdi share that consists of NON-Evrysdi patients = 3%
- 31% of that 3% = **1%** expected to start on the TABLET

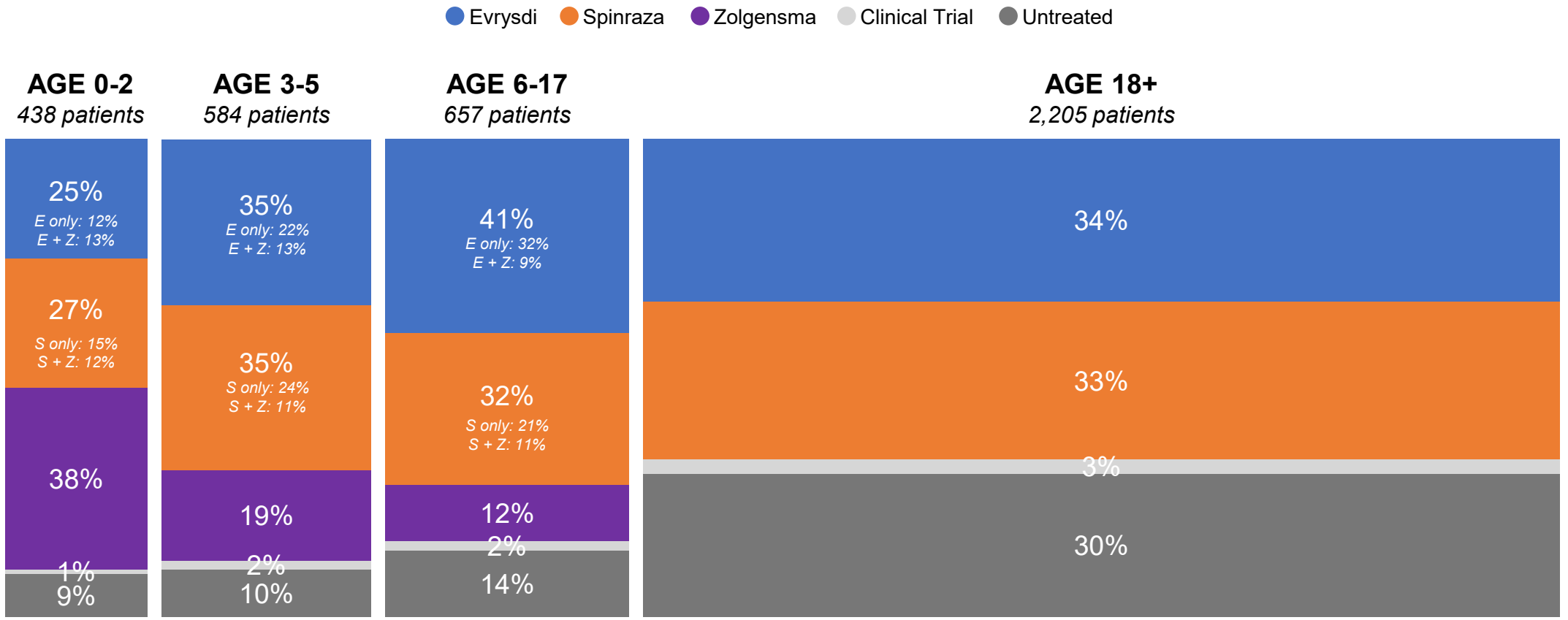
PATIENT TRIANGULATION:
- 21% of Evrysdi patients are expected to convert to the tablet based on Eligibility, bulbar function, and interest in the tablet, aligning with HCP findings.
- 31% of non-Evrysdi patients are eligible for and very interested in the Evrysdi Tablet. When applied to HCP's expected growth coming from this group (+3%), the tablet vs. original formulation proportions align.

CURRENT MARKET SHARE

The market share is strongly influenced by proportion of adult patients, who are nearly evenly split between Evrysdi, Spinraza, and Untreated.

CURRENT SMA TREATMENT SHARE MARIMEKKO

Among Total HCPs (n=100, 3,884 patients represented)



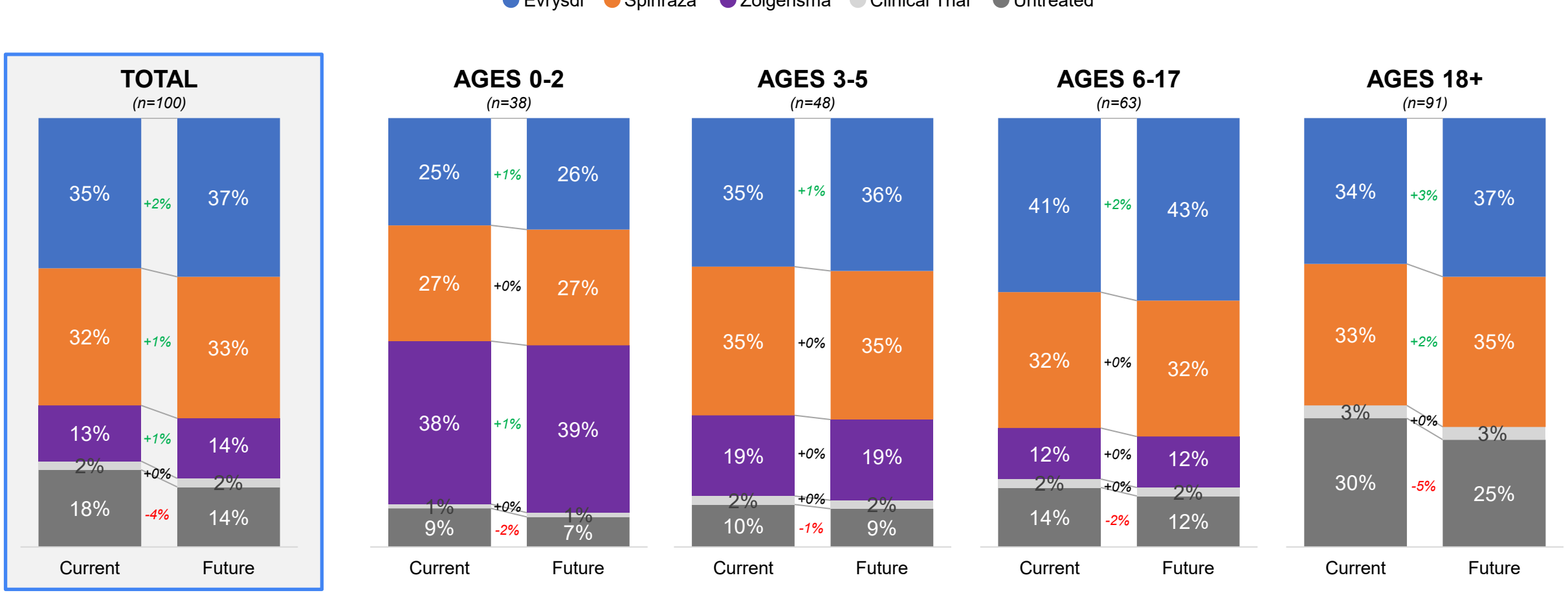
Base: Total HCPs (n=100); B1. Thinking about your spinal muscular atrophy (SMA) patients, what percentage of these patients are currently on the following treatments? Each column must add up to 100%.

FUTURE STATED TREATMENT SHARE WITH NO MARKET CHANGE

HCP stated future shares without any change in the market indicate Evrysdi growth, especially among the older SMA patient population.

FUTURE STATED TREATMENT SHARE: NO CHANGES TO THE MARKET*

Among Total HCPs (n=100)



Base: Total HCPs (n=100)

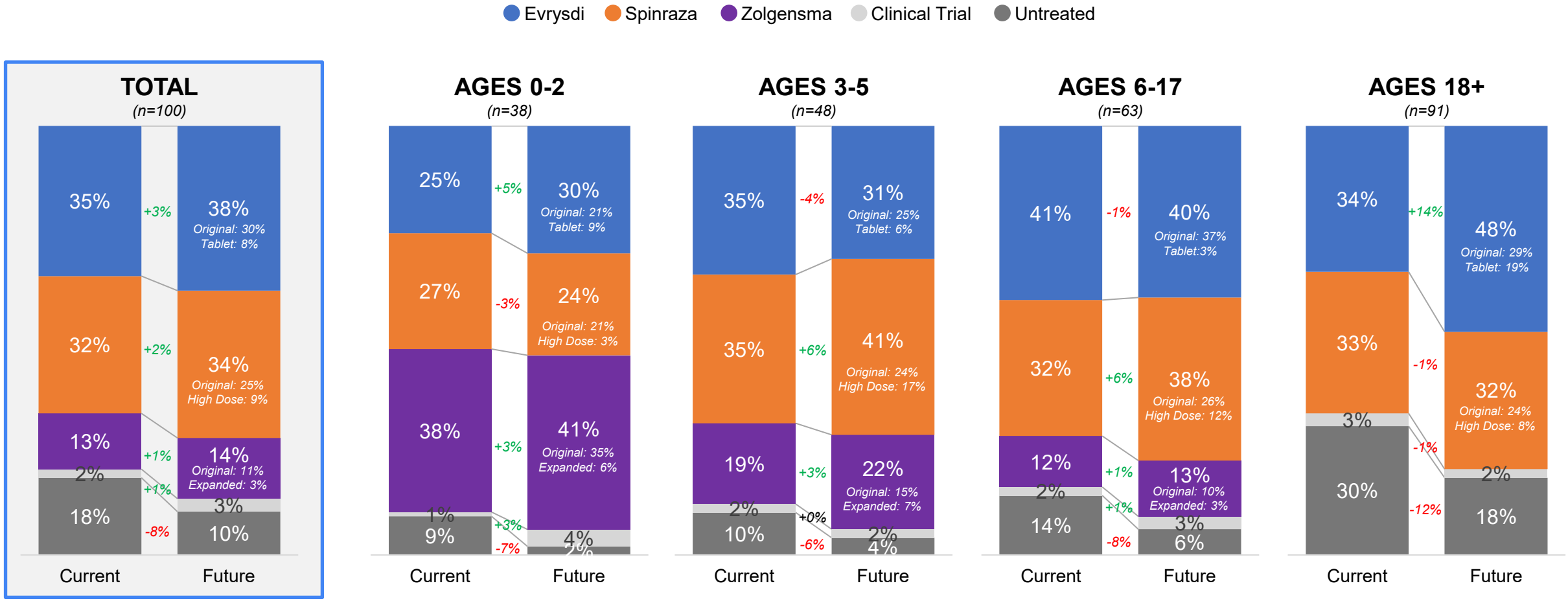
*Note: Future stated shares have been adjusted based on a combination of the HCP stated shares (D12), patient stated future treatment expectations (B5-B6, B9-B10), and patient interest in products in development (F2, F7, F9).

FUTURE TREATMENT SHARE WITH AVAILABILITY OF NEW PRODUCTS

Evrysdi's share increase is highly influenced by 18+ patients who are currently untreated that would start Evrysdi with the introduction of the Tablet.

FUTURE SMA TREATMENT SHARE: EVRYSDI TABLET, HIGH DOSE SPINRAZA, EXPANDED ZOLGENSMA

Among Total HCPs (n=100)
Conjoint Findings



Base: Total HCPs (n=100)

INDIVIDUAL PRODUCT IMPACT ON SMA CONJOINT SHARES

The Evrysdi Tablet provides flexible options; DEVOTE/High Dose Spinraza is time tested; STEER/Expanded Zolgensma benefits from positive patient-reported outcomes.

EVRYSDI TABLET: 10.7%

DRIVERS:
- Provides additional options, flexibility, and fits into their lifestyle
- Perception of comparable efficacy and safety with Evrysdi Original
- Preference from Adults and Untreated SMA patients

DRAWBACKS:
- Tablet dispersion cannot be taken via feeding tube
- Perception of comparable efficacy with Evrysdi Original *(seen both as a driver and a drawback)*

HIGH DOSE SPINRAZA: 12.0%

DRIVERS:
- Clinical data
- Safety
- Doctor recommendation
- Preference from pediatric caregivers (control, routine)

DRAWBACKS:
- Declining Spinraza satisfaction
- Intrathecal injection
- Burdensome administration schedule
- Impact on lower limb function

EXPANDED ZOLGENSMA: 5.4%

DRIVERS:
- One-time administration
- Patient-reported improvement in lower limb motor function, efficacy

DRAWBACKS:
- Indication, limited patient population
- Liver damage
- Lower familiarity and experience with Zolgensma

CONJOINT

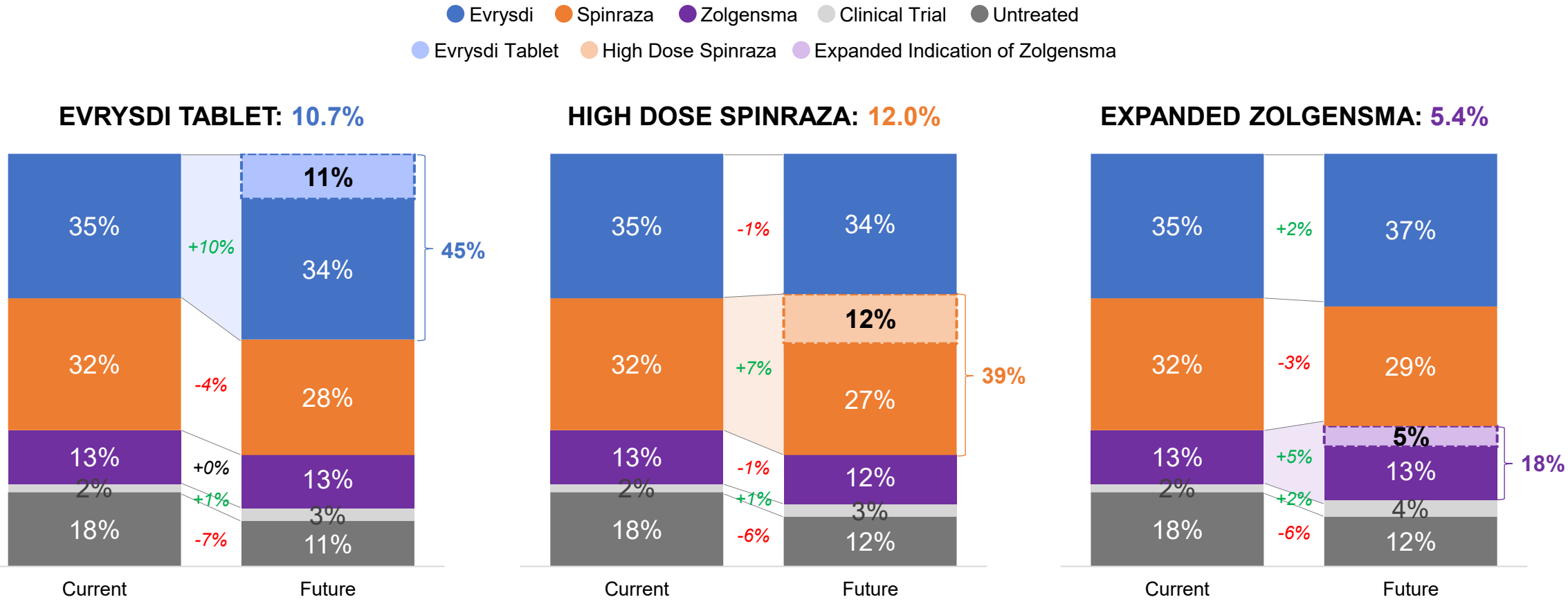
Preference shares are overstated due to new treatment factor, rare disease market where options are limited, overlapping with currently available treatments, thus recommend implementing a deflationary factor of at least 33%. The adult SMA population is heavily influencing the higher share due to their general interest to be treated, which may need further adjustments. In addition, Evrysdi Tablet scenario is indicated for all ages, which contributes to the growth expectation from 0-2, though it's influence on the total share is minimal.

INDIVIDUAL PRODUCT IMPACT ON SMA CONJOINT SHARES

While the introduction of High Dose Spinraza captures the more share than the Evrysdi Tablet when introduced to the market on its own, the Tablet provides a larger expansion of share from currently untreated and Spinraza patients. Spinraza's share lift does not significantly impact Evrysdi's share, with most of the High Dose Spinraza use expected to come from a conversion of current Spinraza patients and expansion from untreated.

FUTURE SMA TREATMENT SHARE WITH AVAILABILITY OF ONE NEW PRODUCT

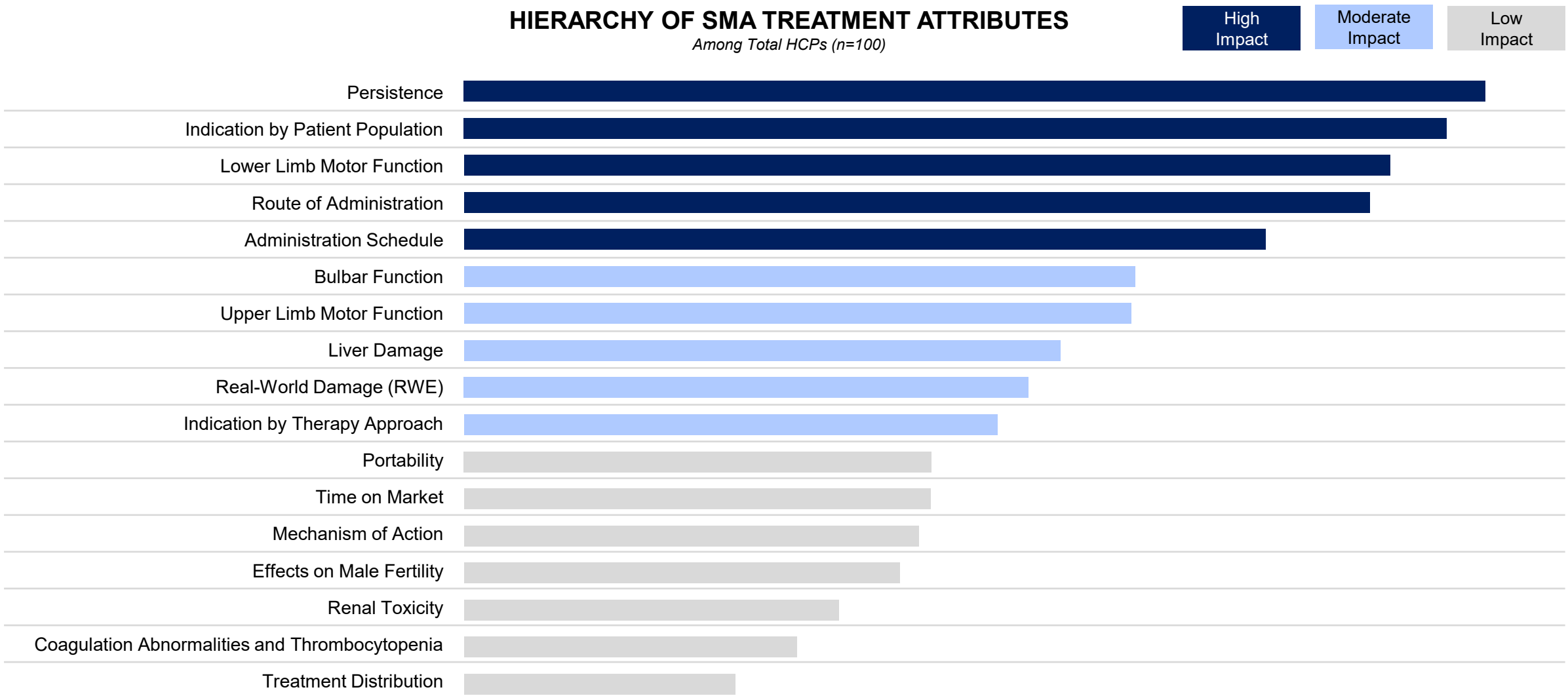
Among Total HCPs (n=100)
Conjoint Findings



Base: Total HCPs (n=100)

HIERARCHY OF SMA CONJOINT ATTRIBUTES

Future SMA treatments must have consistency for better treatment outcomes to a broad SMA patient population with efficacy and convenience. Safety is lower in priority.



AWARENESS AND PERCEPTIONS OF NEW PRODUCTS

PATIENT | HCP

Patient awareness of the Evrysdi Tablet falls significantly short of High Dose Spinraza; however, the impression of the tablet outperforms other products. Interest and likelihood to talk to an HCP about all three products are consistent.

	AWARENESS		IMPRESSION	INTEREST		LIKELIHOOD TO TALK TO HCP
	HCP *7-point scale, rated 3-7*	PATIENT *% Aware*	PATIENT *7-point scale, rated 6-7*	HCP *7-point scale, rated 6-7*	PATIENT *7-point scale, rated 6-7*	PATIENT *7-point scale, rated 6-7*
EVRYSDI TABLET	**83%** *Total HCPs (n=100)*	**34%** *Total Patients (n=100)*	**73%** *Aware of Evrysdi Tablet (n=34)*	**98%** *Aware of Evrysdi Tablet (n=83)*	**45%** *Total Patients (n=100)*	**47%** *Aware of Evrysdi Tablet (n=34)*
HIGH DOSE SPINRAZA	**80%** *Total HCPs (n=100)*	**71%** *Total Patients (n=100)*	**56%** *Aware of High Dose Spinraza (n=71)*	**97%** *Aware of High Dose Spinraza (n=80)*	**44%** *Aware of High Dose Spinraza (n=71)*	**46%** *Aware of High Dose Spinraza (n=71)*
EXPANDED INDICATION OF ZOLGENSMA	**75%** *Total HCPs (n=100)*	**29%** *Total Patients (n=100)*	**45%** *Aware of Expanded Zolgensma (n=29)*	**95%** *Aware of Expanded Zolgensma (n=75)*	**56%** *Aware of Expanded Zolgensma and Under 18 (n=16)*	**50%** *Aware of Expanded Zolgensma and Under 18 (n=16)*

CONJOINT FUTURE PRODUCT SCENARIOS

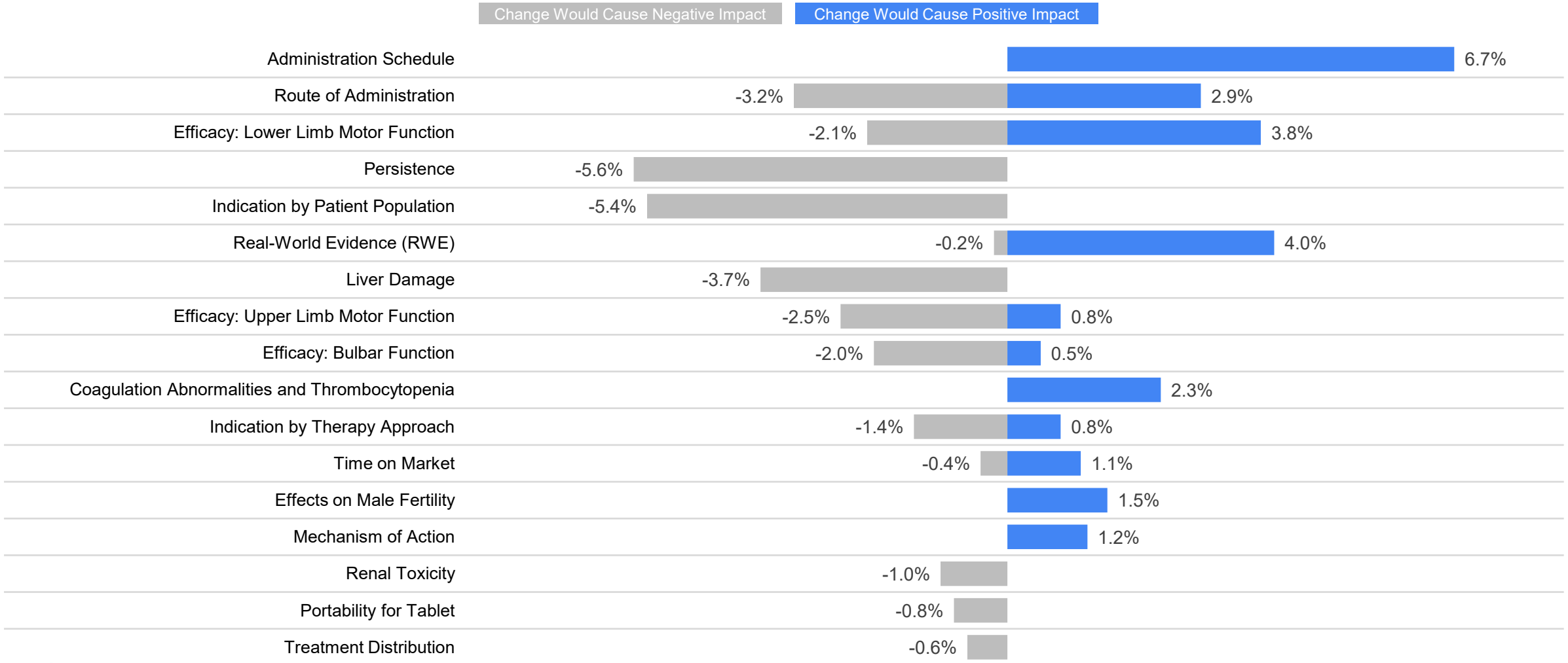
	ATTRIBUTES	TABLET	DEVOTE	STEER
1	Mechanism of Action	Survival of motor neuron 2 (SMN2)- splicing modifier	Survival of motor neuron 2 (SMN2) - directed antisense oligonucleotide	Recombinant AAV9 - based gene therapy
2	Indication by Patient Population	Indicated for SMA patients of all ages (newborn to adults)	Indicated for SMA patients of all ages (newborn to adults)	Indicated for SMA patients 3 to 18 years old
3	Administration Schedule	Daily administration (every day)	Tri-yearly administration (every 4 months)	One time administration
4	Route of Administration	Liquid taken via mouth	Intrathecal injection	Intrathecal injection
5	Portability	Medication is portable at room temperature	Not portable by patient, stored and administered at healthcare facility based on medication guidelines	Not portable by patient, stored and administered at healthcare facility based on medication guidelines
6	Treatment Distribution	Designed to reach the brain, spinal cord, and other areas of the body	Designed to reach the brain, spinal cord, and other areas of the body	Designed to reach the brain, spinal cord, and other areas of the body
7	Time on Market	Available on the market for less than 3 years	Available on the market for less than 3 years	Available on the market for less than 3 years
8	Efficacy: Bulbar Function	Improves bulbar function in infants and children with symptomatic SMA	Improves bulbar function in infants and children with symptomatic SMA	Improves bulbar function in infants and children with symptomatic SMA
9	Efficacy: Upper Limb Motor Function	Patient-reported improvements in upper limb motor function	Patient-reported improvements in upper limb motor function	Patient-reported improvements in upper limb motor function
10	Effects on Male Fertility	Potential effects on male fertility	Data on the effects of male fertility is unavailable	Data on the effects of male fertility is unavailable
11	Coagulation Abnormalities & Thrombocytopenia	Increased risk of coagulation abnormalities and thrombocytopenia	Low risk of coagulation abnormalities and thrombocytopenia	Increased risk of coagulation abnormalities and thrombocytopenia
12	Renal Toxicity	Low risk of renal toxicity	Increased risk of renal toxicity	Low risk of renal toxicity
13	Liver Damage	Low risk of liver damage	Low risk of liver damage	Black box warnings - high risk of liver damage
14	Persistence	90%+ of SMA patients stayed on medication over a 12-month period	50-69% of SMA patients stayed on medication over a 12-month period	90%+ of SMA patients stayed on medication over a 12-month period
15	Real-World Evidence	2-4 years of real-world evidence (RWE) available	5 years + of real-world evidence (RWE) available	6-12 months of real-world evidence (RWE) available
16	Efficacy: Lower Limb Function	Stability in lower limb motor function	Stability in lower limb motor function	Patient-reported improvements in lower limb motor function
17	Indication by Therapy Approach	Indicated as monotherapy - single medication only	Indicated as monotherapy - single medication only	Indicated as monotherapy - single medication only

TABLET CONJOINT SENSITIVITY

Generally, most attributes are fixed for Evrysdi Tablet, however Real-World Evidence (RWE) of 5+ years will have a positive impact on Tablet.

THE EVRYSDI TABLET ATTRIBUTE SENSITIVITIES TORNADO CHART

Among Total HCPs (n=100)
Conjoint Findings



Base: Total HCPs (n=100)

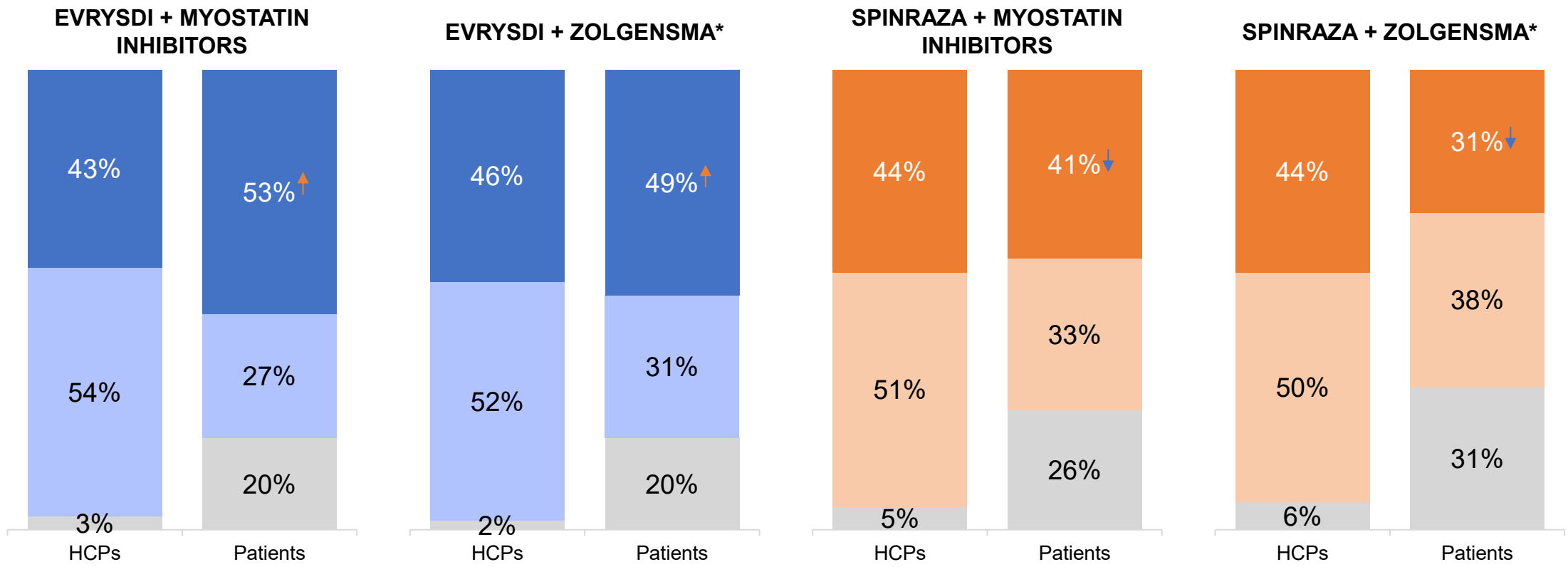
COMBINATION THERAPY INTEREST

PATIENT | HCP

There is interest in combination therapies, particularly among HCPs. SMA Patients/Caregivers have a stronger interest for combination with Evrysdi (+Myostatin Inhibitors and +Zolgensma).

INTEREST IN COMBINATION THERAPIES

Among Total HCPs (n=100) and Total Patients (n=100)
7-Point Scale



Base: Total HCPs (n=100) F2. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, how interested are you in prescribing the following SMA treatment combinations in the future?
Base: Total Patients (n=100) H1. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, how interested are you in the following SMA treatment combinations in the future?
*Note: Only caregivers were asked interest level in Evrysdi and Spinraza in combination with Zolgensma (n=35)

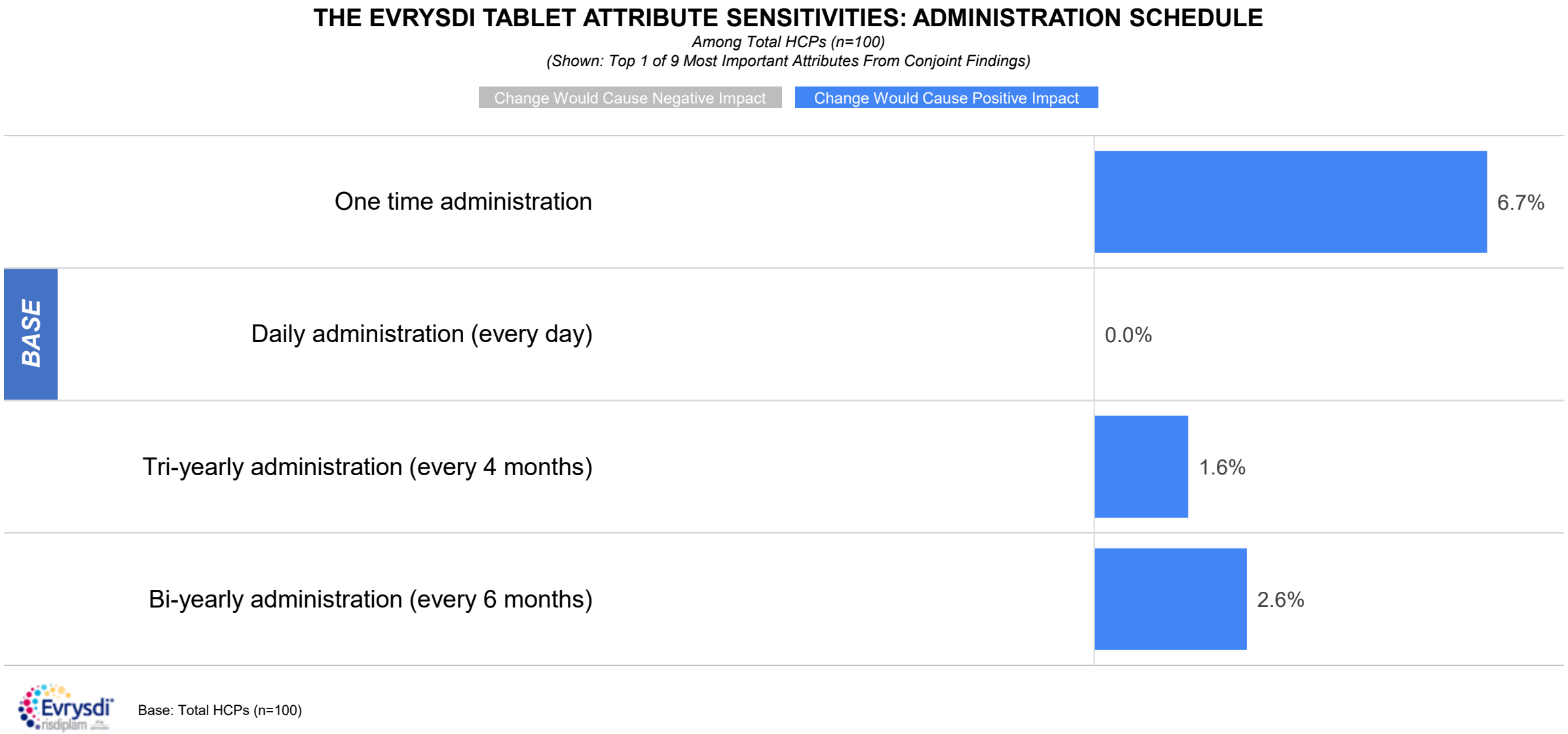
DETAILED FINDINGS

HCPs: TABLET ATTRIBUTE IMPORTANCE

CONJOINT FINDINGS

TABLET ATTRIBUTE SENSITIVITIES: ADMINISTRATION SCHEDULE

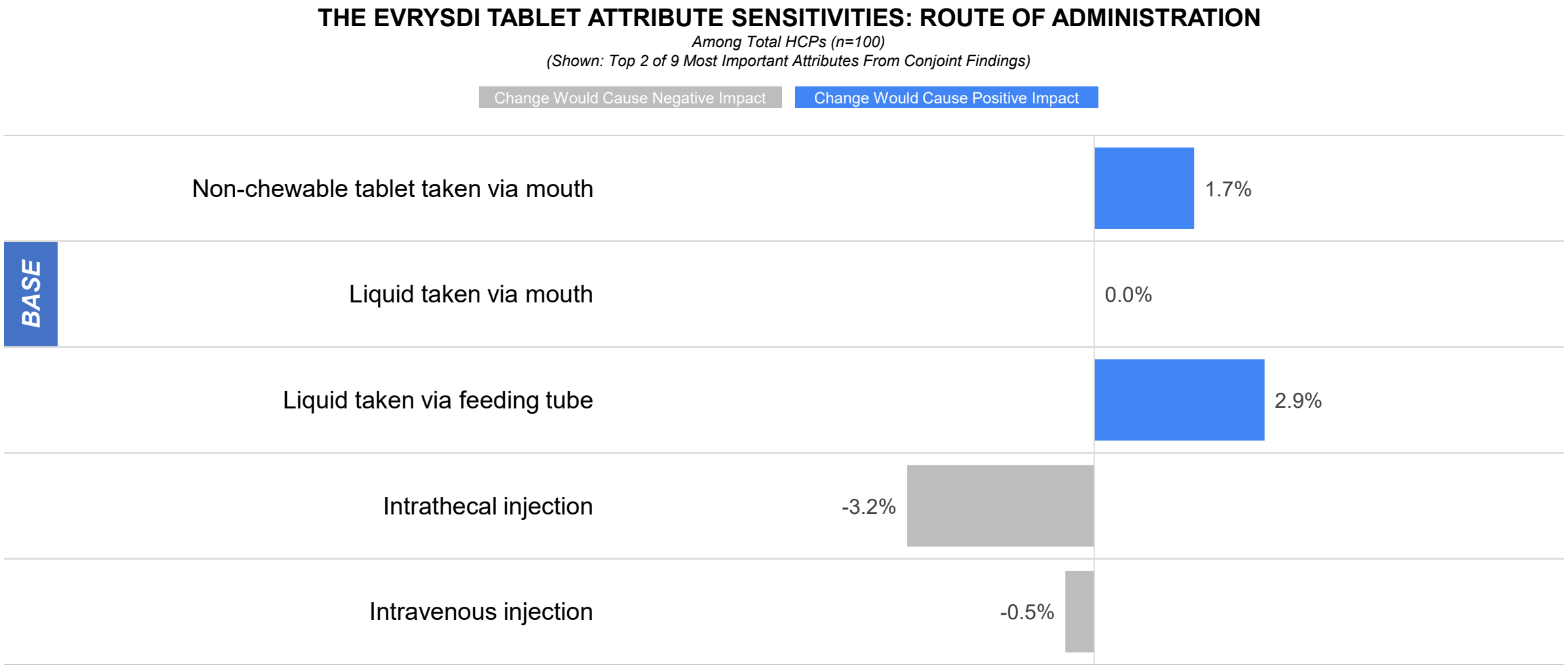
Less frequent administration is preferred but daily administration is acceptable and does not impact share (positive or negative.)



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: ROUTE OF ADMINISTRATION

Non-injectable routes of administration is preferred. Liquid via feeding tube provides the most flexibility for SMA Patients.



Base: Total HCPs (n=100)

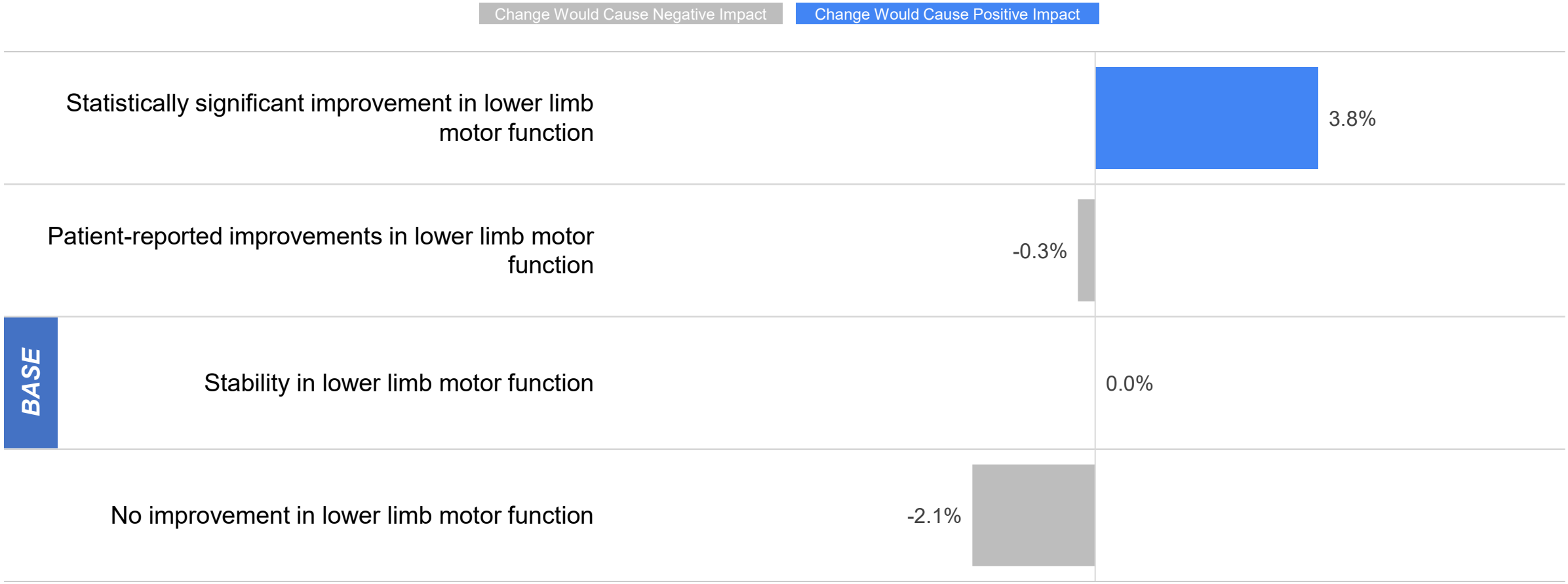
TABLET ATTRIBUTE SENSITIVITIES: LOWER LIMB MOTOR FUNCTION

Only statistically significant improvement in lower limb function (positive impact) or no improvement in lower motor function (negative impact) will have an impact on share.

THE EVRYSDI TABLET ATTRIBUTE SENSITIVITIES: EFFICACY: LOWER LIMB MOTOR FUNCTION

Among Total HCPs (n=100)

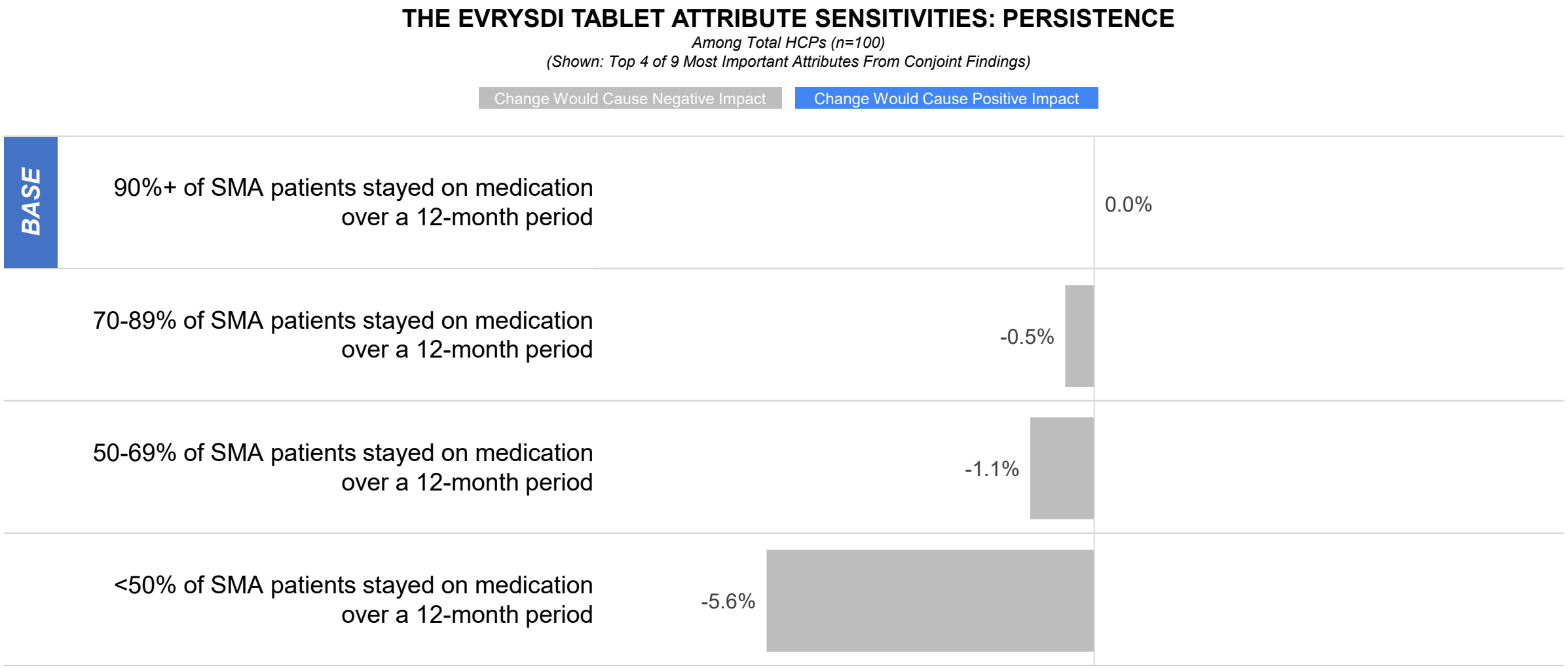
(Shown: Top 3 of 9 Most Important Attributes From Conjoint Findings)



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: PERSISTENCE

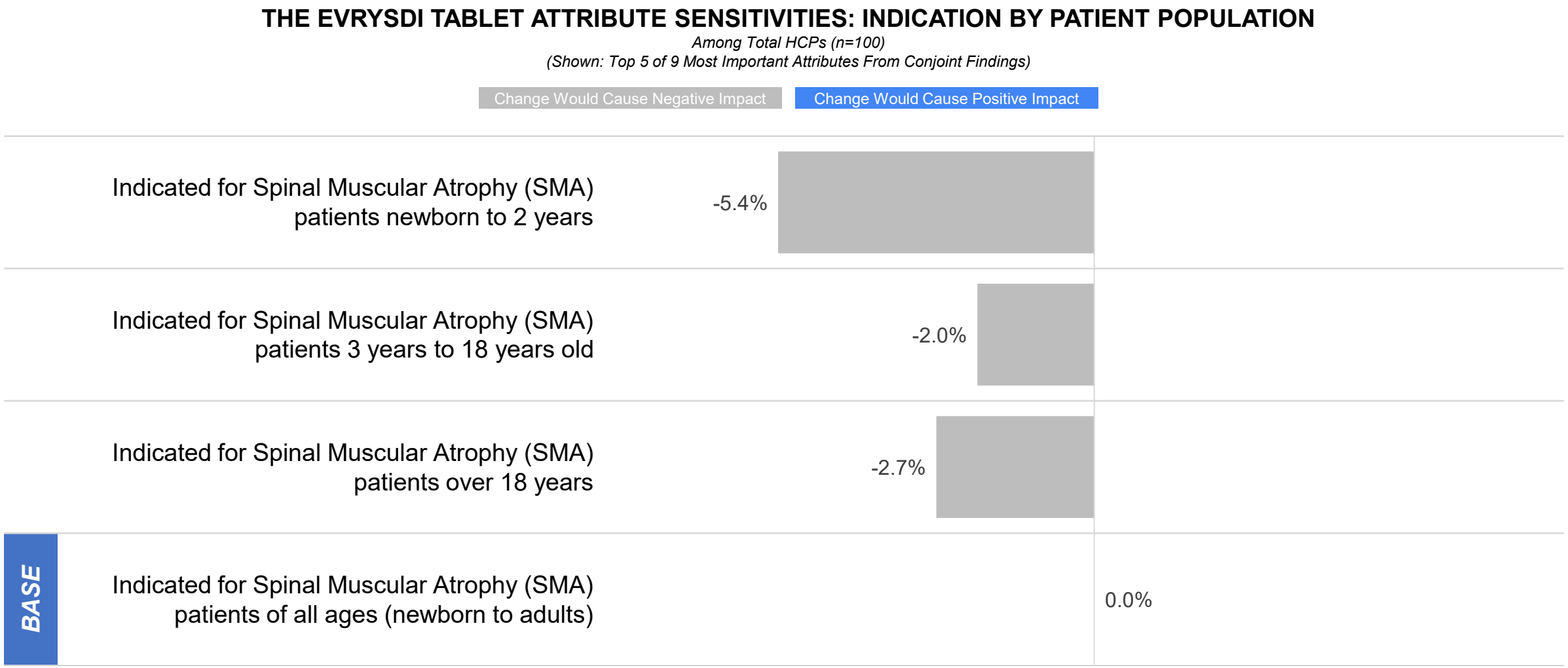
Persistence is strong in SMA – if less than 50%, significant negative impact on share.



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: INDICATION BY PATIENT POPULATION

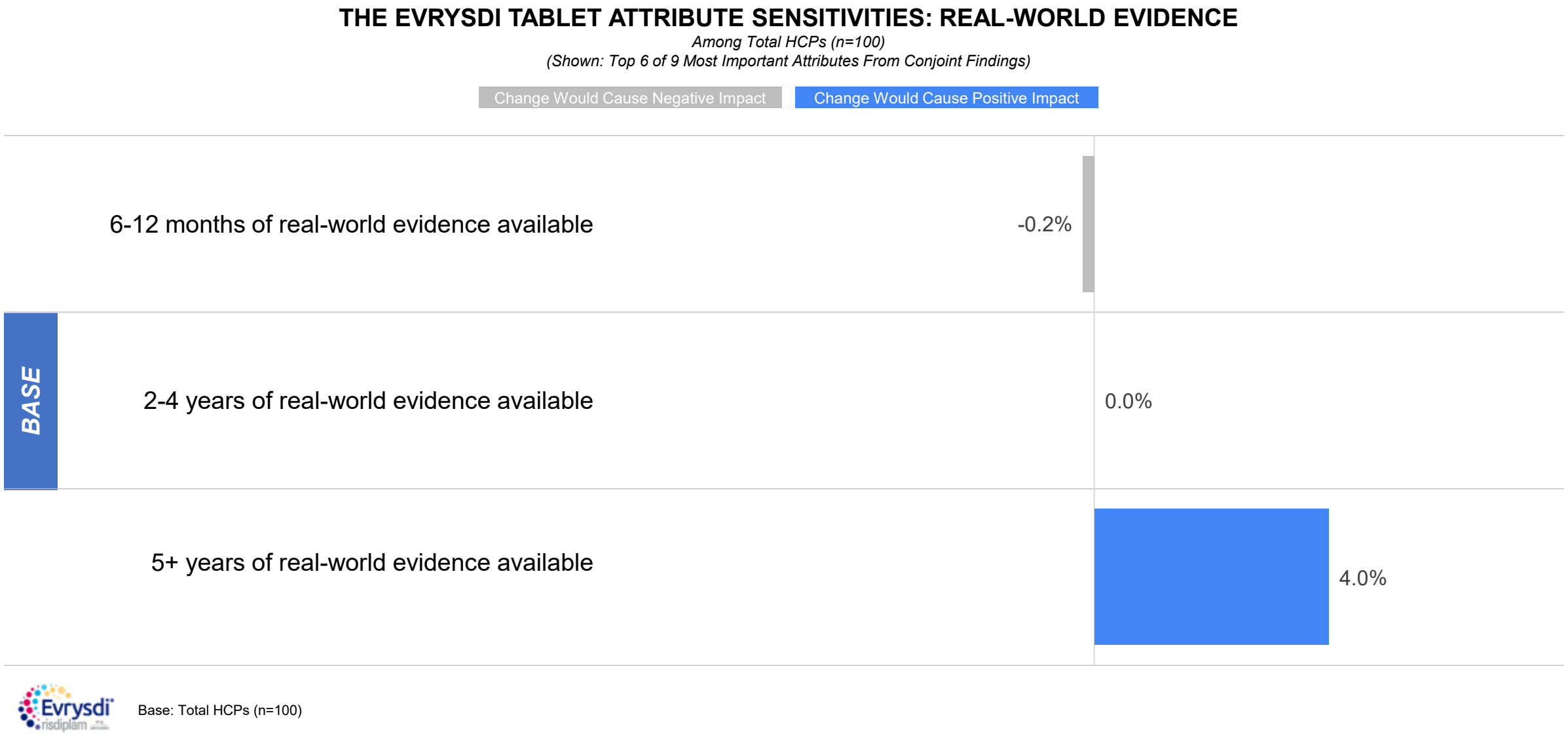
Broader patient indication is preferred.



Base: Total HCPs (n=100)

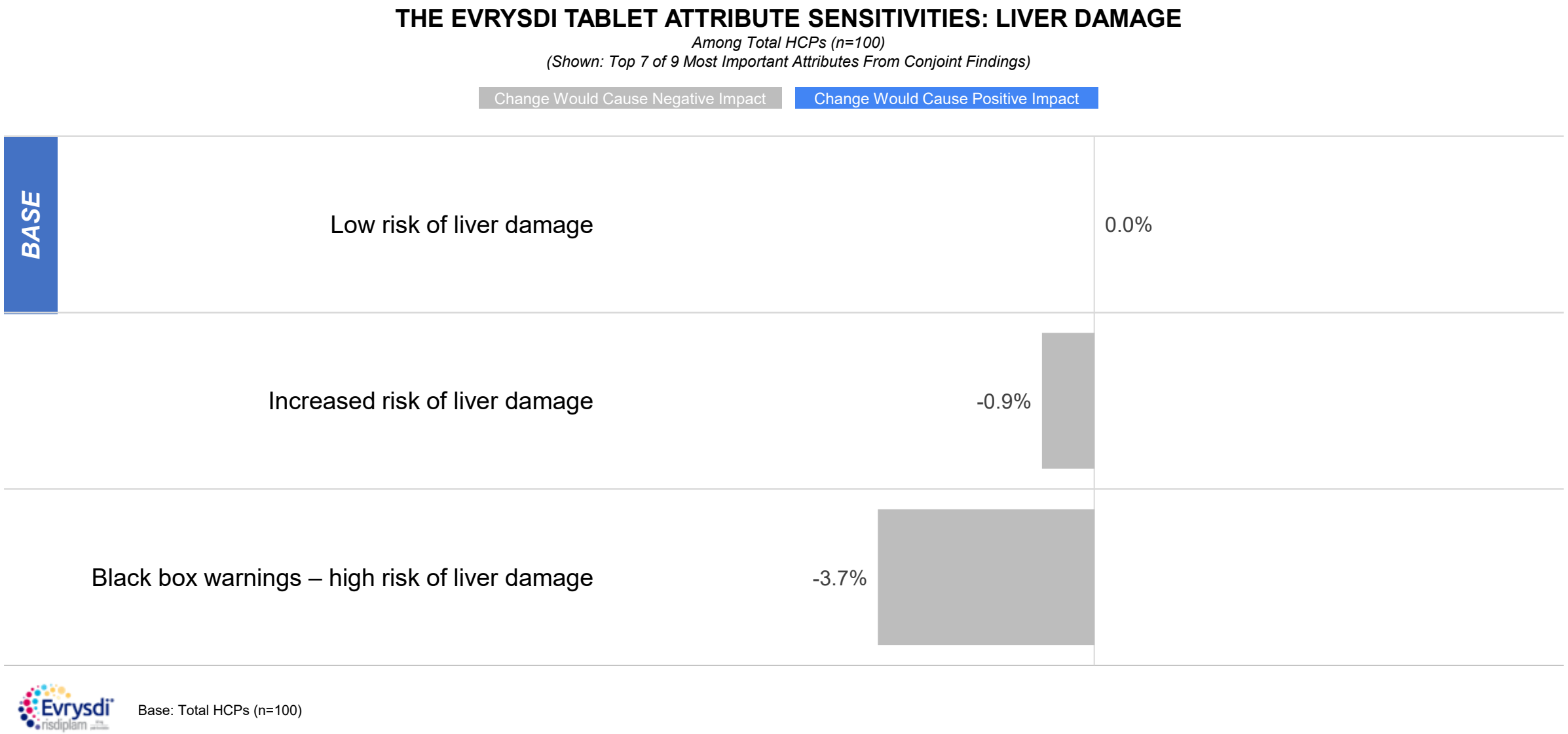
TABLET ATTRIBUTE SENSITIVITIES: REAL-WORLD EVIDENCE

Longer available real-world evidence is preferred. Evrysdi Tablet shares will increase as the RWE increases.



TABLET ATTRIBUTE SENSITIVITIES: LIVER DAMAGE

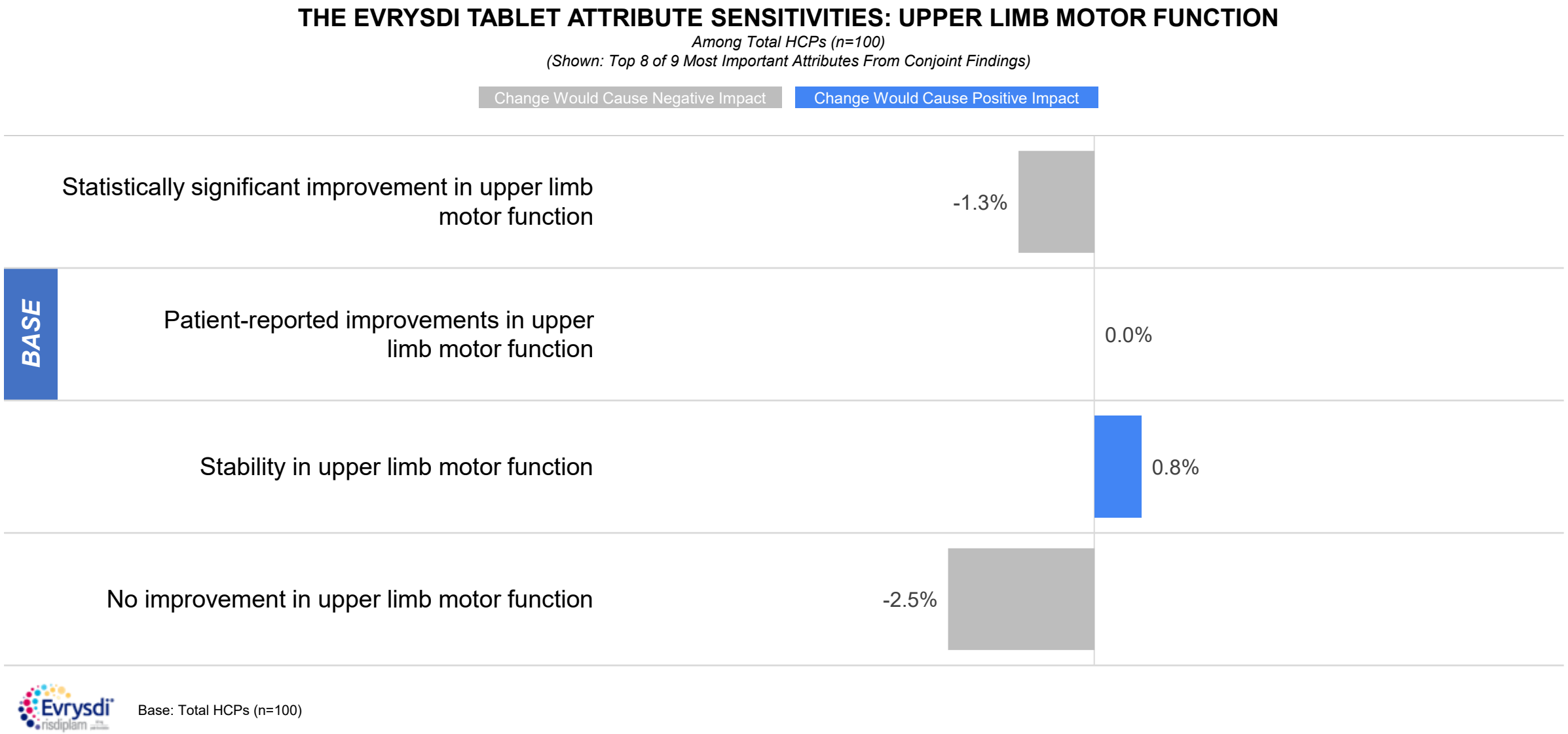
Some liver damage is acceptable, but black box warnings will have a negative impact on share.



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: UPPER LIMB MOTOR FUNCTION

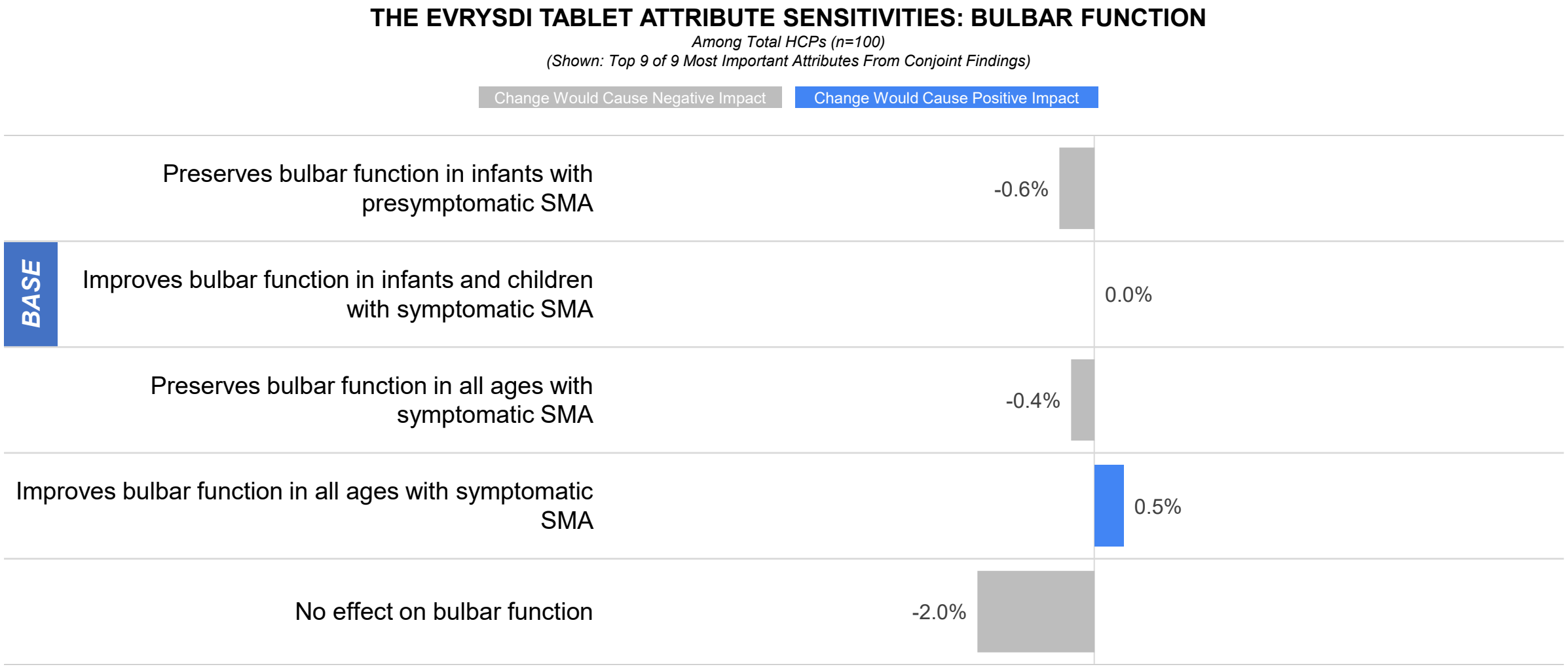
Stability in upper limb function has a positive impact on share, while mentions of improvement in motor function yield a negative impact on share.



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: BULBAR FUNCTION

Expectations that bulbar function is preserved or improved for a new SMA product. High threshold to have a positive impact on share – bulbar function improvement in all ages.



Base: Total HCPs (n=100)

DETAILED FINDINGS

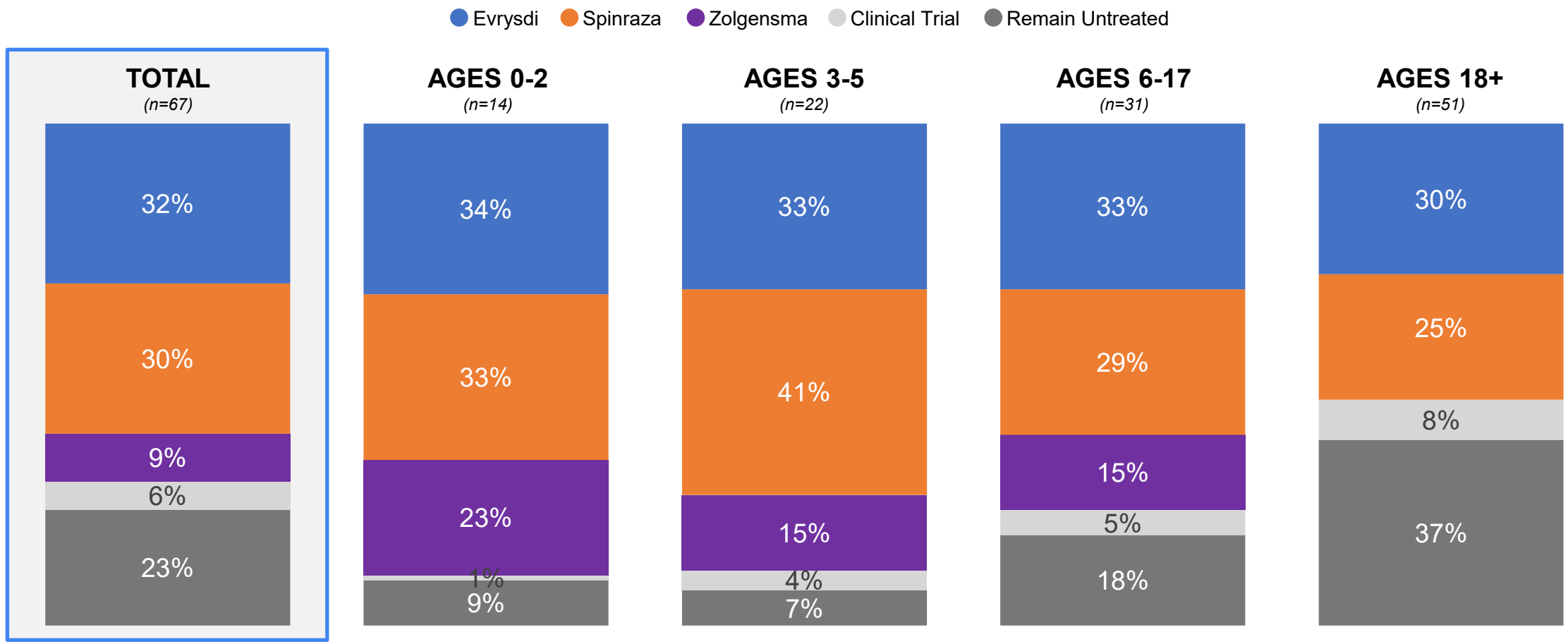
HCPs: TREATMENT SHARE

FUTURE STATED TREATMENT FOR TREATMENT NAÏVE PATIENTS

HCPs state strong future movement towards treatment for their currently treatment naïve patients, with new starts remaining consistent with the current market share proportions.

FUTURE STATED TREATMENT EXPECTATION FOR TREATMENT NAÏVE PATIENTS

Among HCPs with Treatment Naïve Patients (n=67)



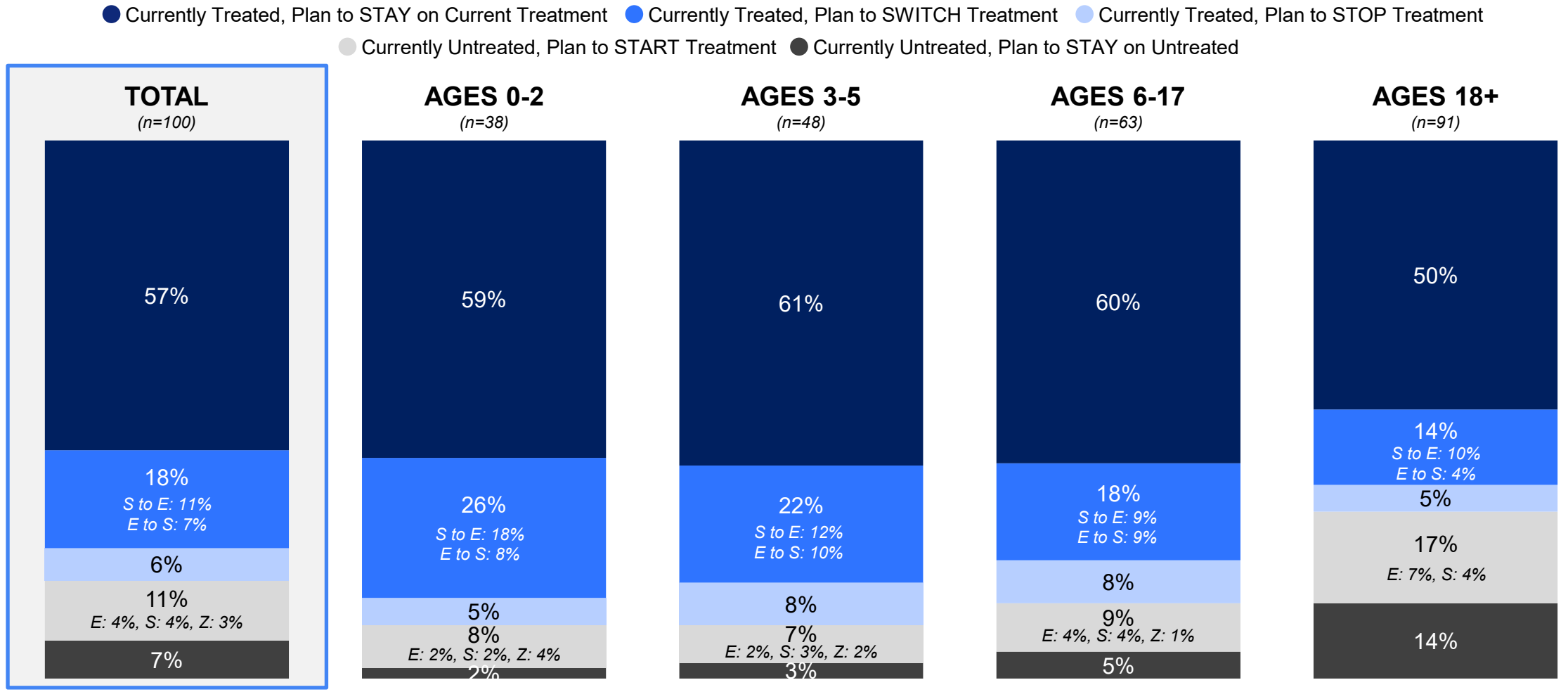
Base: HCPs with Treatment Naïve Patients (n=67)
B7A. Now, thinking about your spinal muscular atrophy (SMA) patients that have NEVER been treated, what percentage of these patients do you anticipate being treated with the following treatments in the next 12 months?

FUTURE TREATMENT EXPECTATION WITH NO MARKET CHANGE: HCP

HCPs anticipate that nearly two thirds of their patients will remain on their current treatment regimen or remain untreated. More patients are expected to switch or start on Evrysdi than switch off of or discontinue, further supporting the expectation of a future Evrysdi share growth.

FUTURE TREATMENT EXPECTATION WITH NO MARKET CHANGE

Among Total HCPs (n=100)



Base: Total HCPs (n=100)

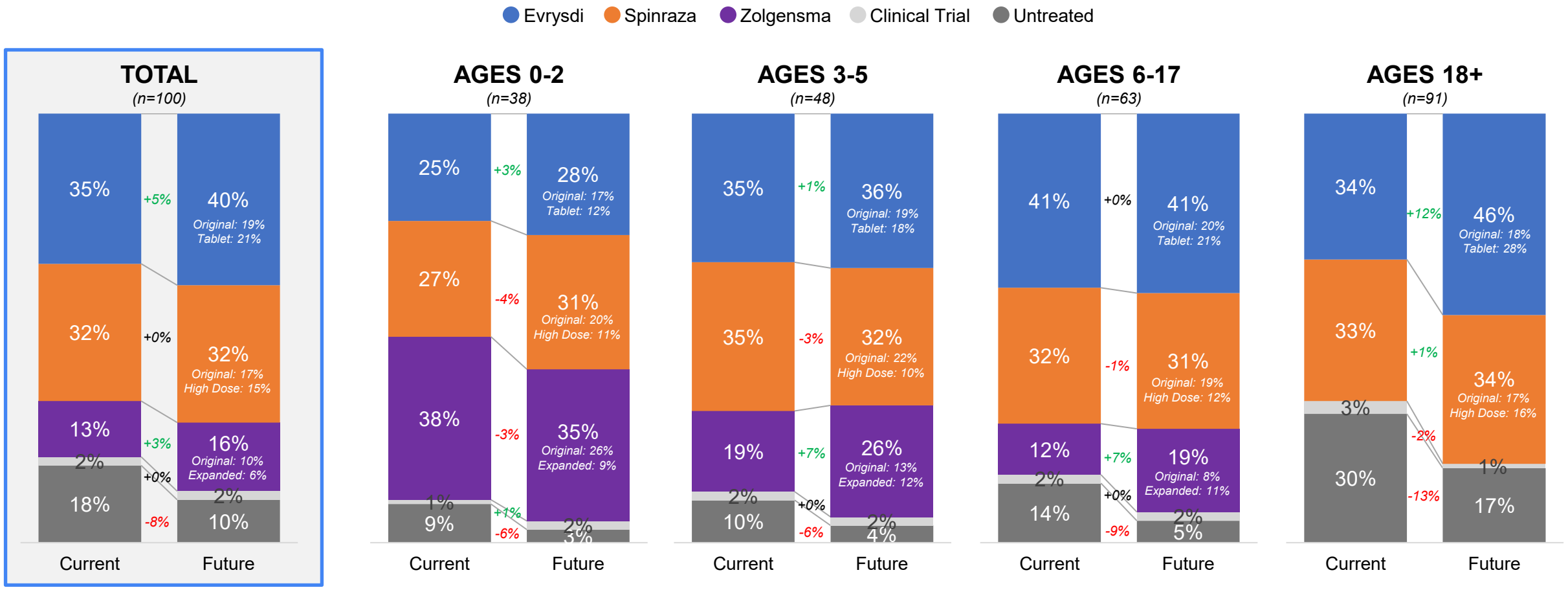
B8. Please assume this hypothetical situation: You see 5 SMA patients in the upcoming year that fall under each of the following age groups. For each age group, how many patients would you anticipate fall into the following treatment categories? Each column total must add up to 5. B9. Among the [INSERT SUM OF B8r2] SMA patients you would plan to initiate SMA treatment, how many would be initiated on the following? B10. Among the [INSERT SUM OF B8r4] SMA patients you would plan to switch from their current SMA treatment, how many would be switched to the following?

FUTURE STATED TREATMENT SHARE WITH AVAILABILITY OF NEW PRODUCTS

The HCP stated future treatment share provides additional confirmation and support for the derived shares extrapolated from the conjoint scenario analysis.

FUTURE STATED TREATMENT SHARE: EVRYSDI TABLET, HIGH DOSE SPINRAZA, EXPANDED ZOLGENSMA*

Among Total HCPs (n=100)



Base: Total HCPs (n=100)

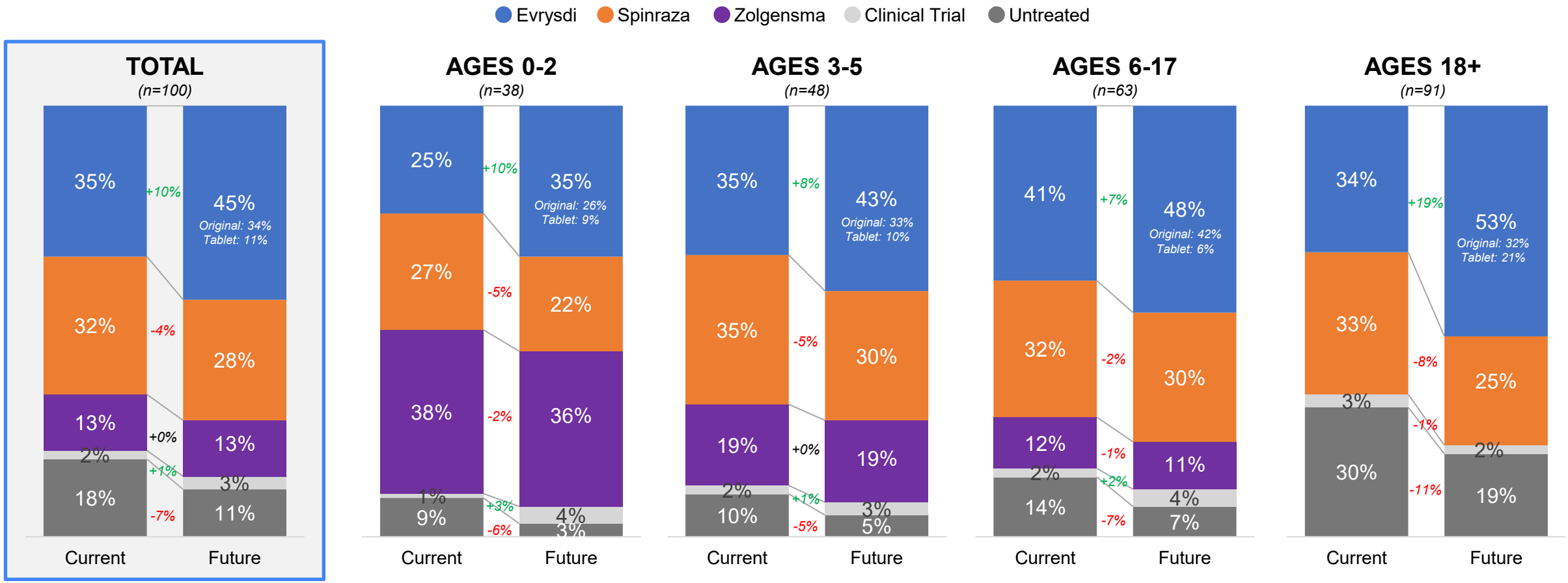
*Note: Future stated shares have been adjusted based on a combination of the HCP stated shares (D12), patient stated future treatment expectations (B5-B6, B9-B10), and patient interest in products in development (F2, F7, F9).

FUTURE TREATMENT SHARE WITH AVAILABILITY OF EVRYSDI TABLET

With the scenario of the Evrysdi Tablet being the only available new product on the market, shares are expected to increase significantly, especially among 18+ patients. Shares would primarily be taken from currently untreated patients as well as current Spinraza users.

FUTURE SMA TREATMENT SHARE WITH EVRYSDI TABLET AVAILABILITY

Among Total HCPs (n=100)
Conjoint Findings



Base: Total HCPs (n=100)

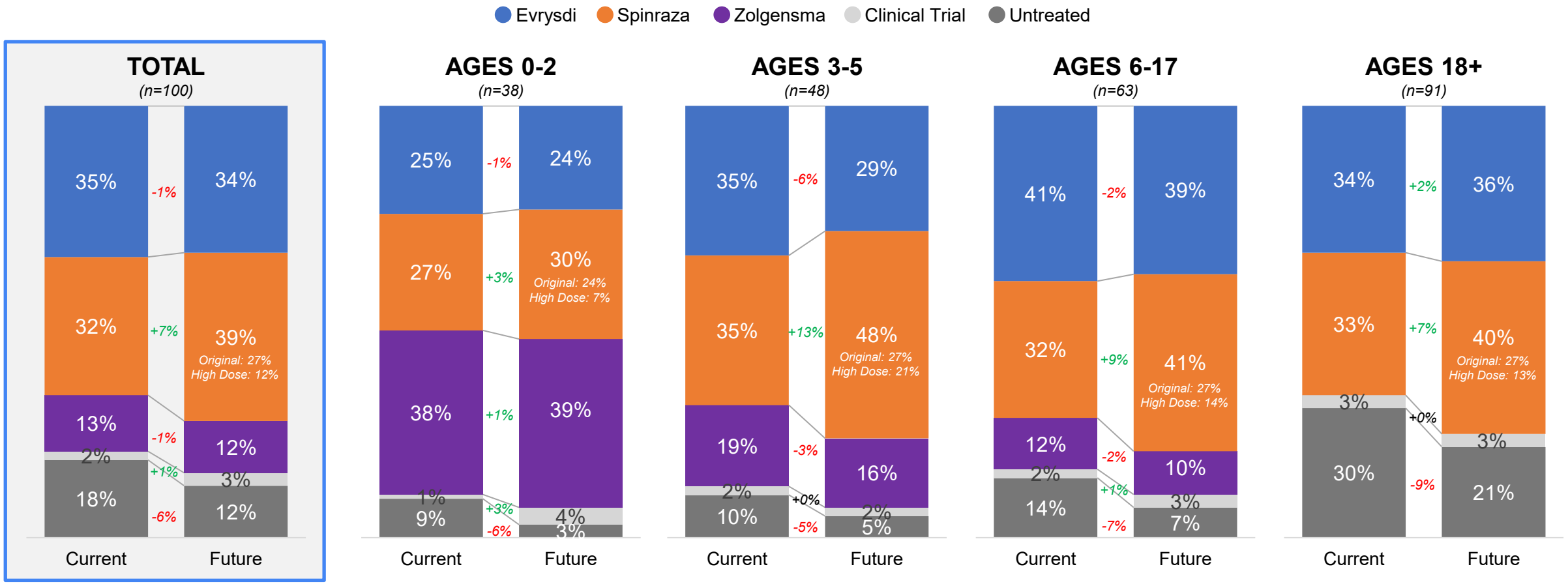
FUTURE TREATMENT SHARE WITH AVAILABILITY OF HIGH DOSE SPINRAZA

HCP

With the scenario of High Dose Spinraza being the only available new product on the market, shares are expected to moderately increase. Evrysdi share would be minimally impacted with the new product introduction, as the growth in shares would primarily come from untreated patients.

FUTURE SMA TREATMENT SHARE WITH HIGH DOSE SPINRAZA AVAILABILITY

Among Total HCPs (n=100)
Conjoint Findings



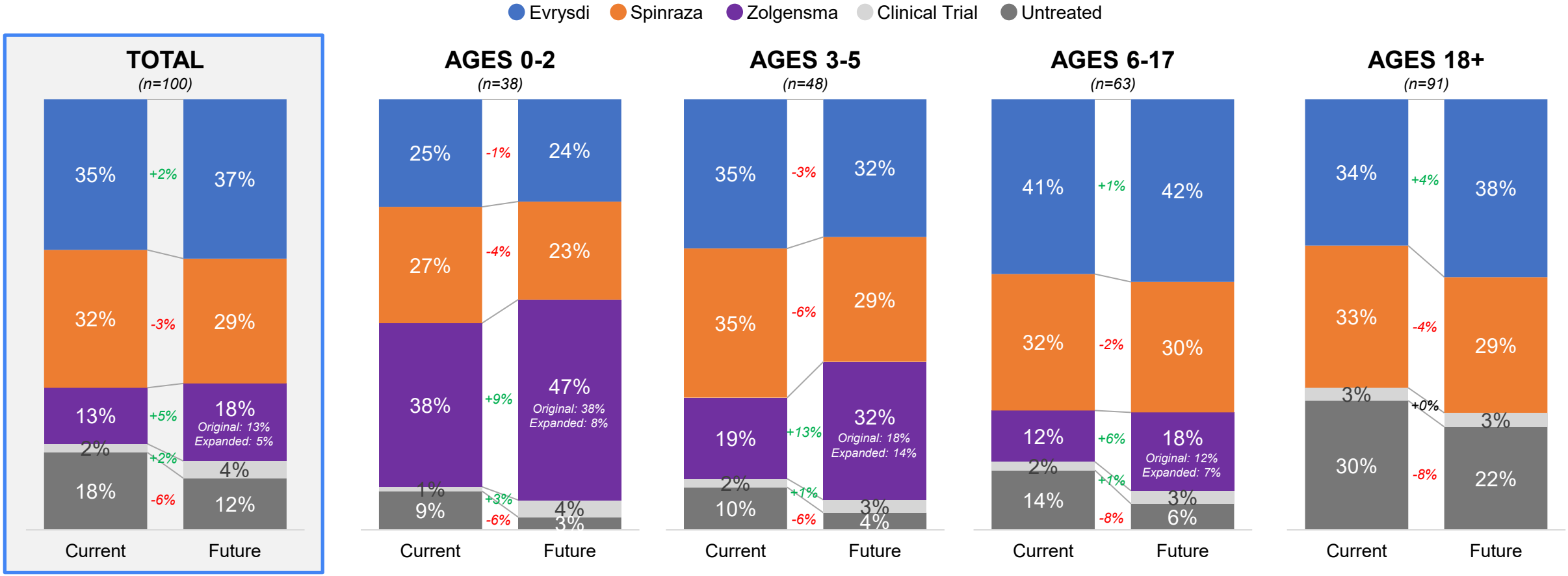
Base: Total HCPs (n=100)

FUTURE TREATMENT SHARE WITH AVAILABILITY OF EXPANDED INDICATION OF ZOLGENSMA

With the scenario of the Expanded Indication of Zolgensma being the only available new product on the market, Zolgensma shares are expected to moderately increase. Evrysdi share would not be impacted, as shares would primarily come from current Spinraza users and untreated patients ages 0-17.

FUTURE SMA TREATMENT SHARE WITH EXPANDED INDICATION OF ZOLGENSMA AVAILABILITY

Among Total HCPs (n=100)
Conjoint Findings



Base: Total HCPs (n=100)

DETAILED FINDINGS

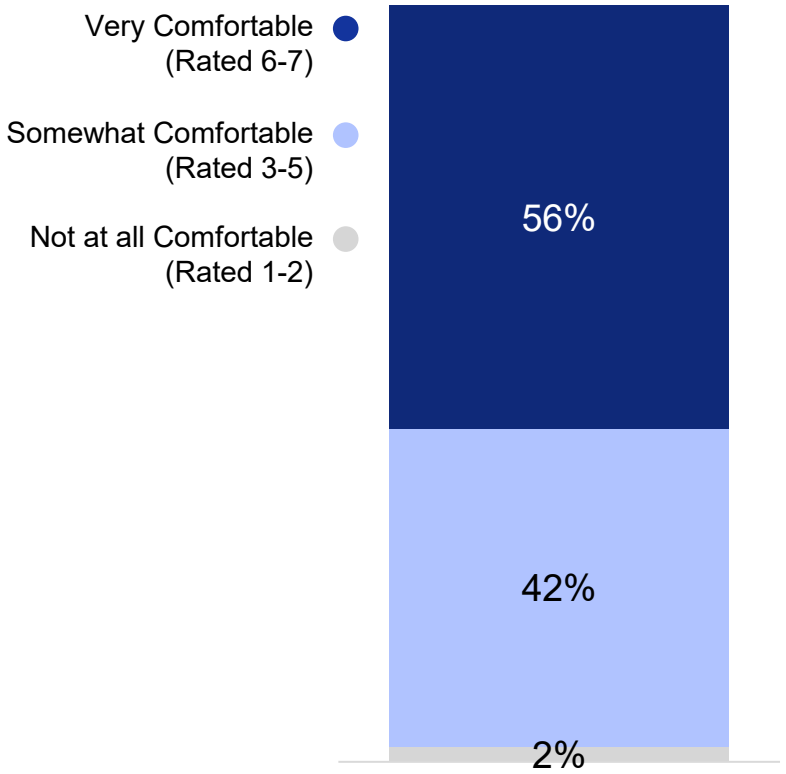
HCPs: CURRENT TREATMENT PERCEPTIONS

PRESCRIBING PERCEPTIONS

Only half of physicians feel very comfortable treating their SMA patients. Overall, there is a lot of interest in combination therapy, with 4 in 10 physicians indicating high levels of interest in these treatment approaches.

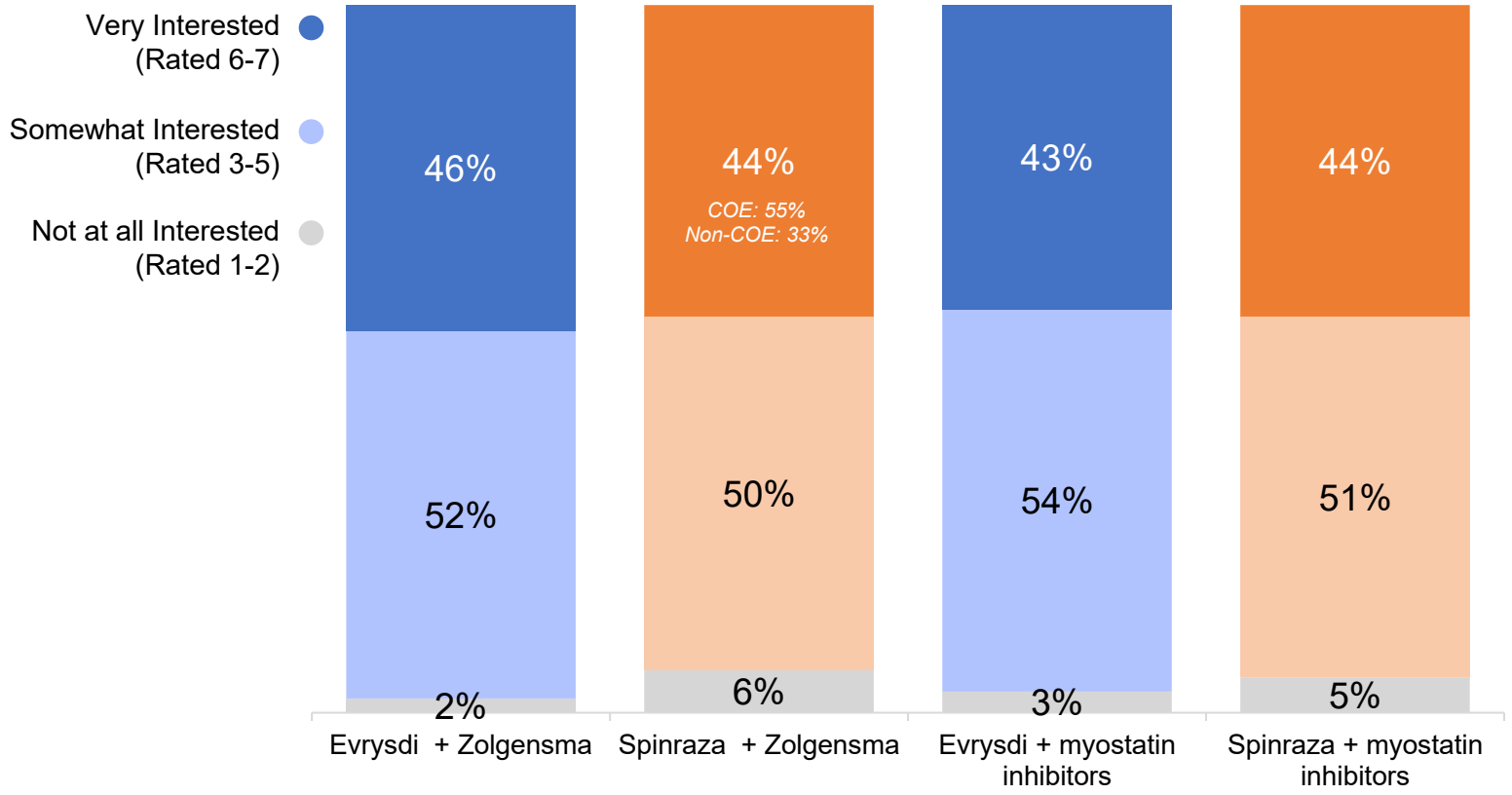
COMFORT TREATING SMA PATIENTS

Among Total HCPs (n=100)
7-Point Scale



INTEREST IN PRESCRIBING COMBINATION THERAPIES

Among Total HCPs (n=100)
7-Point Scale



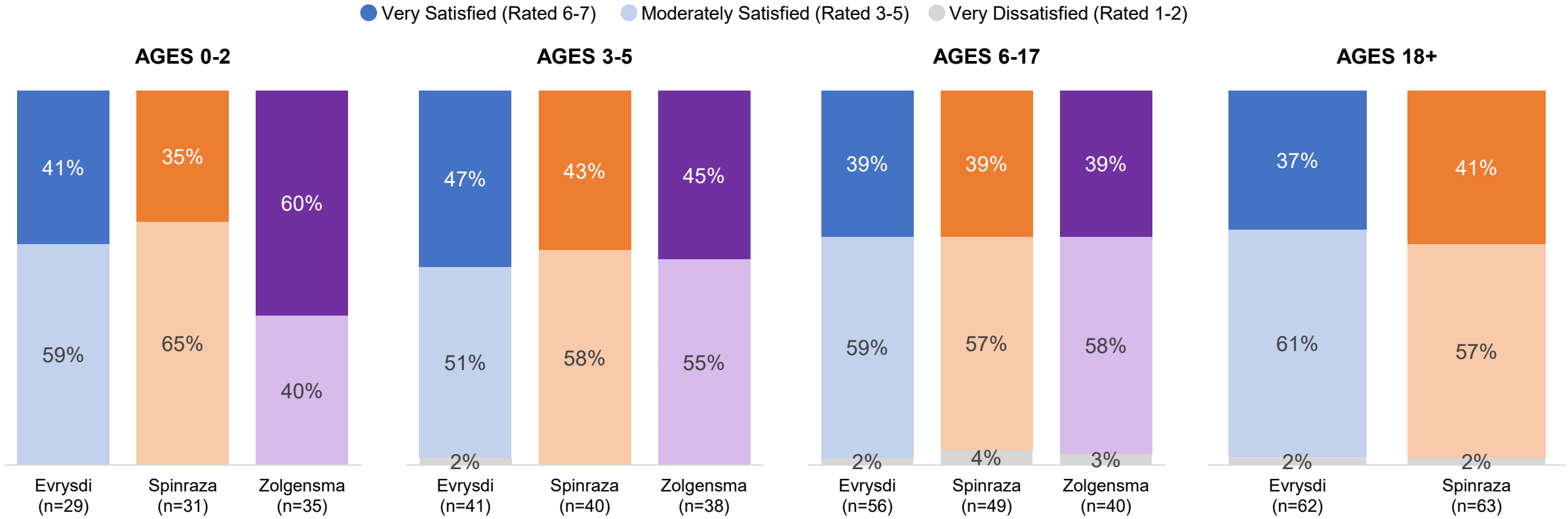
Base: Total HCPs (n=100)
F1. Using a 7-point scale, where 1=Not At All Comfortable and 7=Extremely Comfortable, how comfortable are you with managing and treating SMA?
F2. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, how interested are you in prescribing the following SMA treatment combinations in the future?

TREATMENT SATISFACTION

No significant differences in satisfaction with SMA treatments across patient age groups, with the large majority being moderately-to-extremely satisfied with all three DMTs. Unsurprisingly, there is a directional increase in satisfaction with Zolgensma in patients ages 0-2-year-old.

TREATMENT SATISFACTION BY PATIENT AGE GROUP

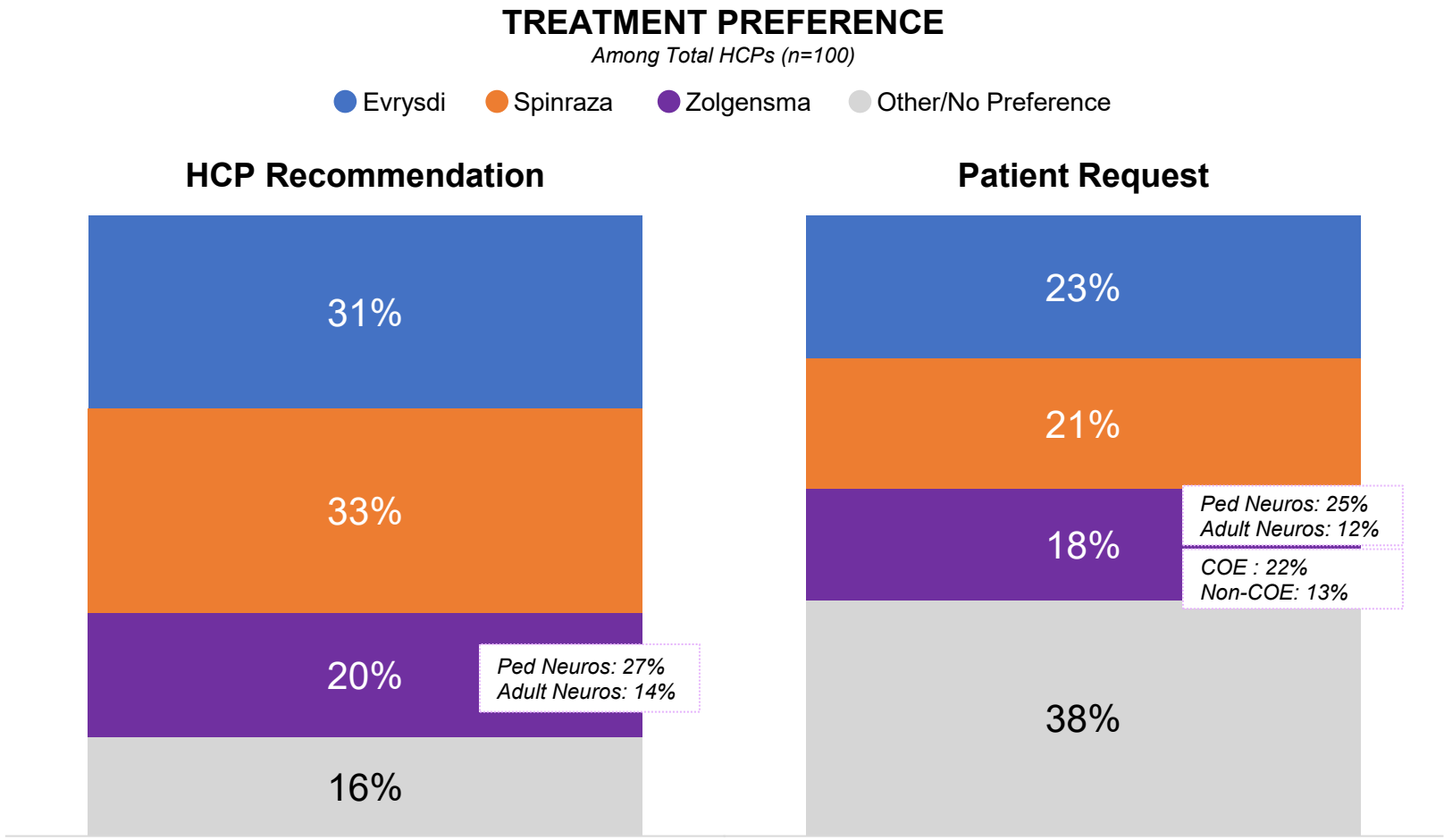
Among HCPs who Treat Patients in Each Age Group
7-Point Scale



Base: Total HCPs (n=100); Pediatric Neurologists (n=43), Adult Neurologists (n=57)
B2. Using a 7-point scale, where 1=Not At All Satisfied and 7=Extremely Satisfied, what is your level of satisfaction with the following spinal muscular atrophy (SMA) treatments by the different age categories?

TREATMENT REQUESTS AND RECOMMENDATIONS

Preference for Spinraza and Evrysdi is comparable in both physicians and patients, as indicated by HCPs. While physicians fulfill specific patient requests around half the time, discrepancies in doctor and patient treatment preference are less common (20% of the time).



HCPs prescribe a patient's specific treatment request *49%* of the time on average.

An HCP recommendation and a patient request differ *20%* of the time on average.

Base: Total HCPs (n=100)
B3. What percent of the time do you recommend the following spinal muscular atrophy (SMA) treatments to your SMA patients? / B5. What percent of your spinal muscular atrophy (SMA) patients request the following SMA treatments? / B6. Among those spinal muscular atrophy (SMA) patients that request a specific SMA treatment, what percent of the time do you prescribe the SMA product that the patient requests? / B7. What percent of the time do your spinal muscular atrophy (SMA) patients have a treatment preference that differs from your treatment preference?

DETAILED FINDINGS

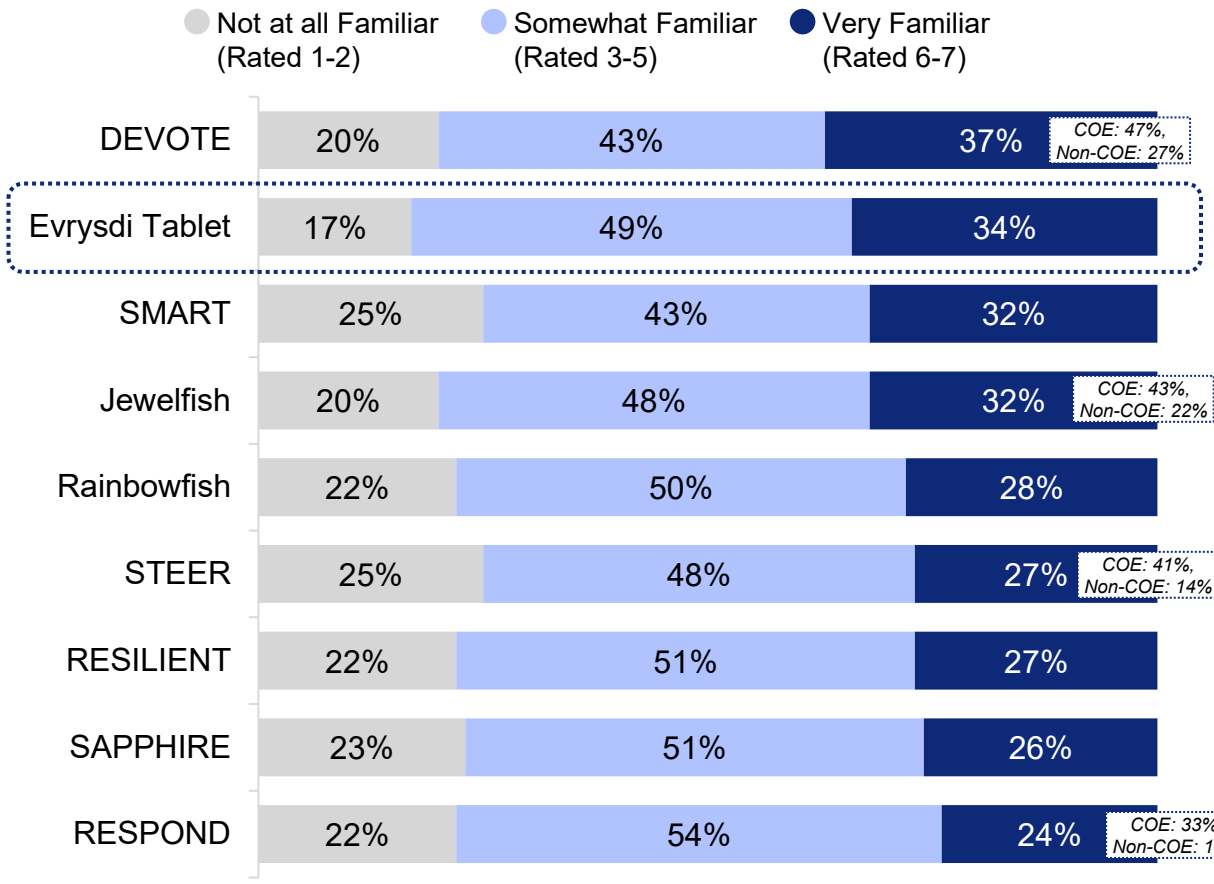
HCPs: TREATMENTS IN DEVELOPMENT

CLINICAL TRIAL FAMILIARITY AND INTEREST

Majority of HCPs (83%) are somewhat-to-very familiar with the Evrysdi Tablet – comparable to familiarity with DEVOTE. HCP interest in the Evrysdi Tablet is comparable to other trials, with the highest interest being in the Jewelfish trial.

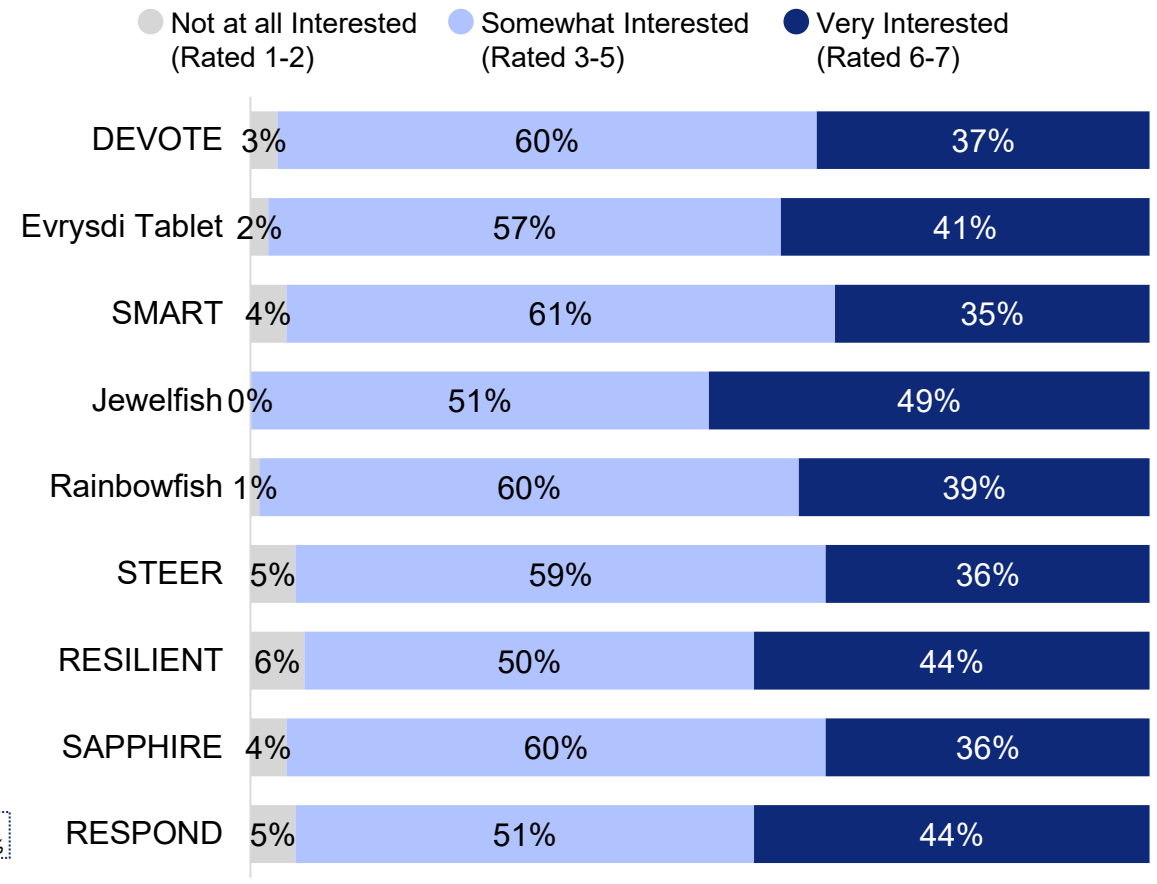
FAMILIARITY WITH CLINICAL TRIALS

Among Total HCPs (n=100)
7-Point Scale



INTEREST IN CLINICAL TRIALS

Among HCPs Familiar with Clinical Trial (sample size varies)
7-Point Scale



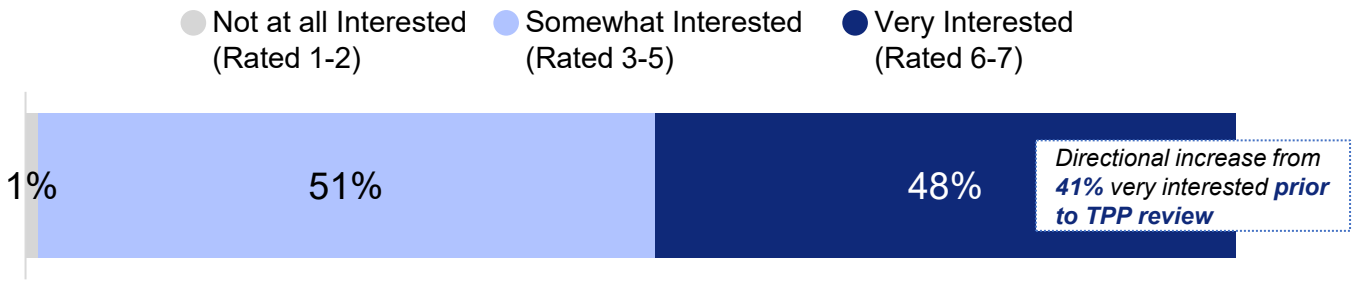
Base: Total HCPs (n=100) D8. Using a 7-point scale, where 1=Not At All Familiar and 7=Extremely Familiar, what is your level of familiarity with the following clinical trials?
Base: Familiar of Trial (Evrysdi Tablet n=83, DEVOTE n=80, Rainbowfish n=78, Jewelfish n=80, SMART n=75, RESPOND n=78, STEER n=75, RESILIENT n=78, SAPPHIRE n=77)
D9. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, what is your level of interest with the following clinical trials?

EVRYSDI TABLET PRESCRIBING INTEREST

Following review of the Evrysdi Tablet product profile, there is a directional increase in interest to prescribe (+7%). Ninety percent of HCPs would prescribe the Evrysdi Tablet within a year after receiving approval.

INTEREST IN PRESCRIBING EVRYSDI TABLET

(Following Review of TPP)
Among Total HCPs (n=100)
7-Point Scale



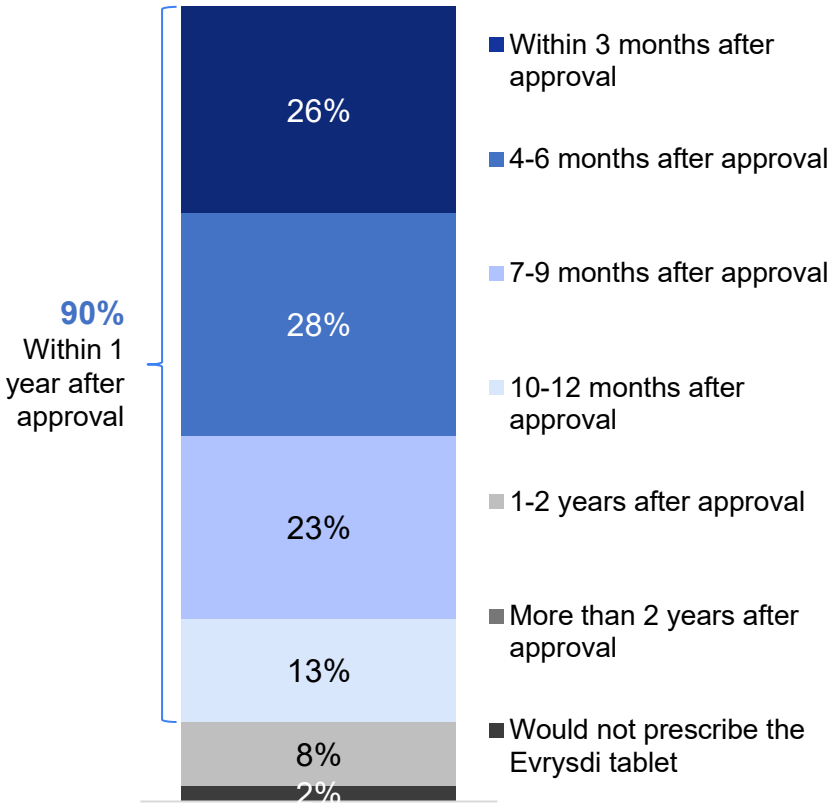
*"It may be more **convenient and compact to store** than liquid for older patients."*
- Pediatric Neurologist

*"It would be great for patients who can swallow pills. Also, it can be mixed so once on fixed amount, **anyone can use.**"*
- Pediatric Neurologist

*"Some patients will prefer a tablet over a liquid, **may improve compliance.**"*
- Adult Neurologist

TIMELINE OF TABLET PRESCRIBING

Among Total HCPs (n=100)
% Selected



Base: Total HCPs (n=100) D1. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, what is your level of interest in prescribing the Evrysdi tablet? / D2A. Why are you NOT interested in prescribing the Evrysdi tablet? / D2B. Why are you interested in prescribing the Evrysdi tablet? / D6. Assuming that the Evrysdi tablet became FDA approved, how long after approval would you begin prescribing the Evrysdi tablet to your SMA patients?

TABLET ADVANTAGES AND MOTIVATION

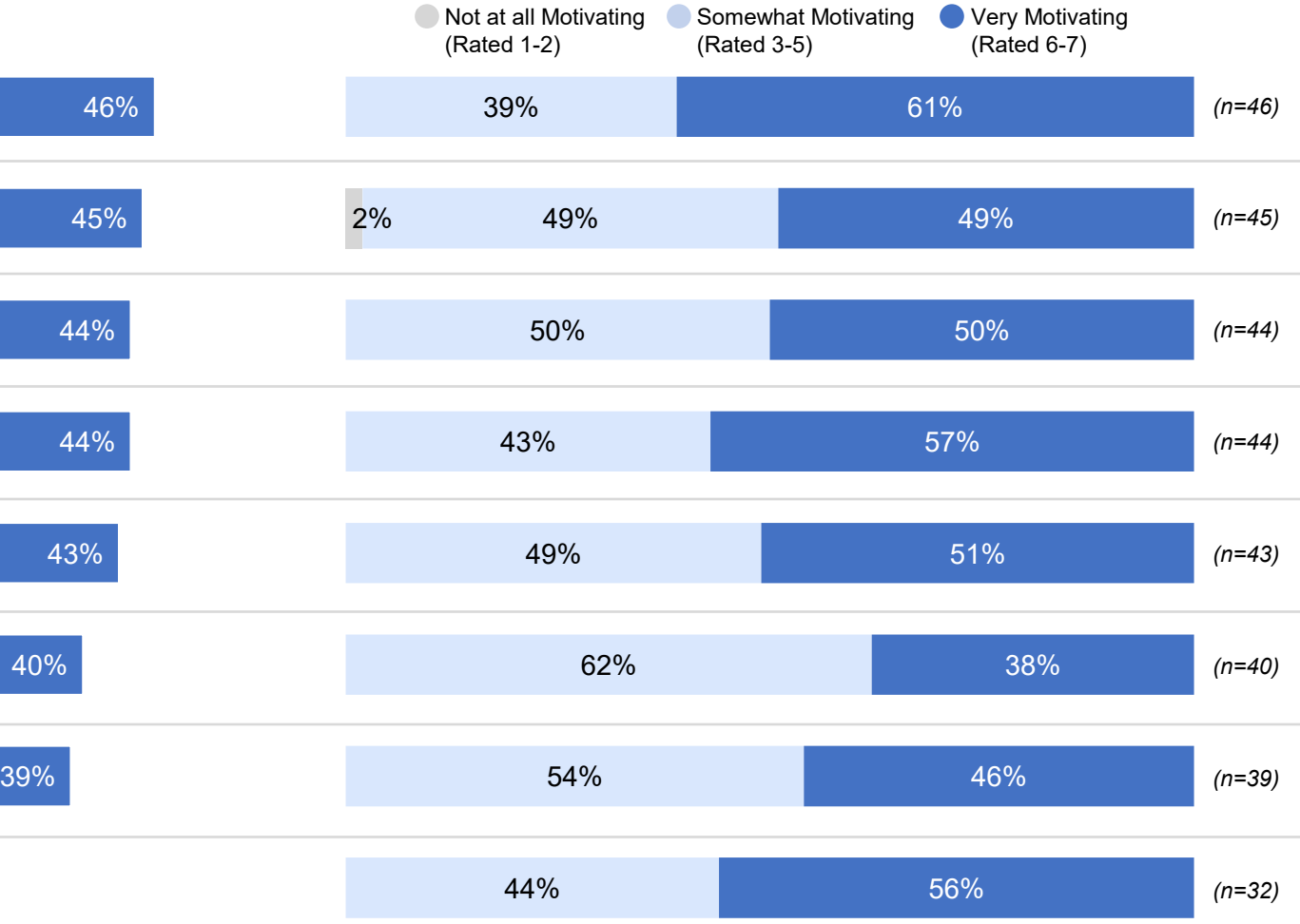
Leading advantages to the Evrysdi Tablet include room temperature storage, patient eligibility requirements, and the additional options the formulation provides patients. Of the tablet's advantages, room temperature storage is the most motivating for HCPs to prescribe.

TOP PERCEIVED EVRYSDI TABLET ADVANTAGES

Among Total HCPs (n=100)
% Selected

MOTIVATION TO PRESCRIBE

7-point scale



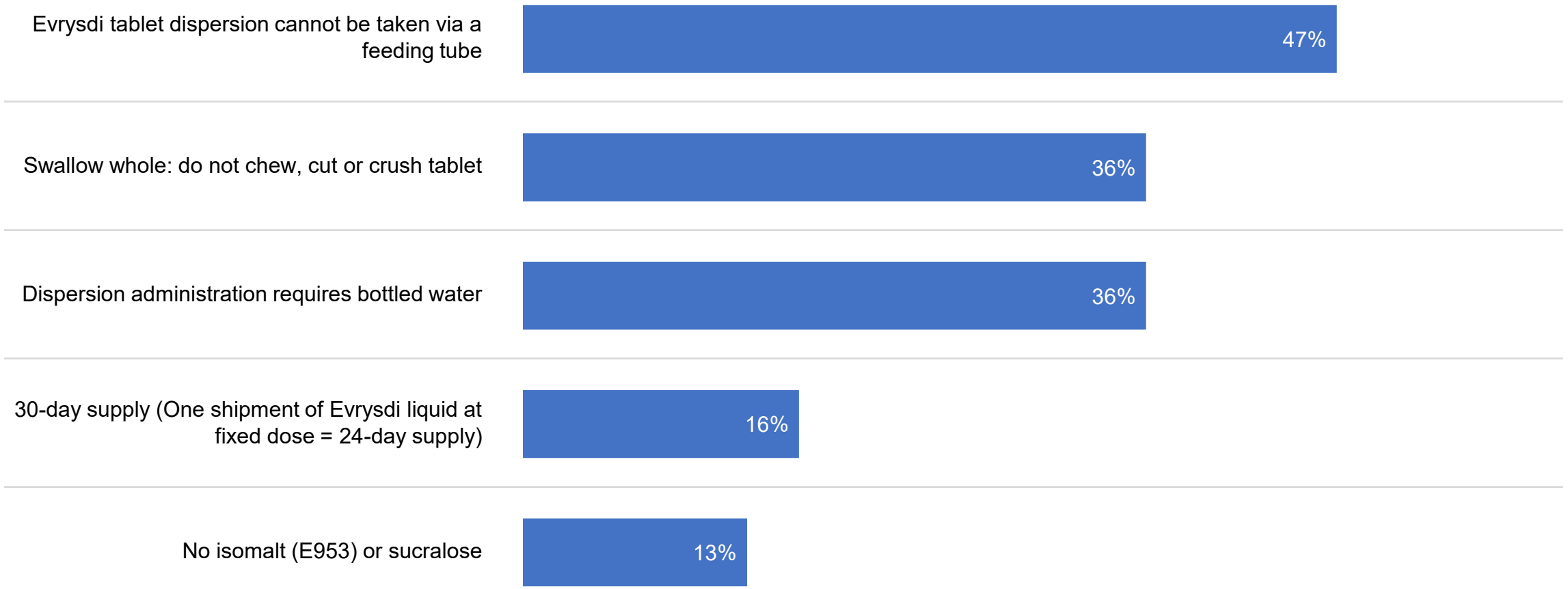
Base: Total HCPs (n=100) D3. What are the primary advantages of the Evrysdi Tablet for you? Please select up to 5 features in each column.
Base: Identified Attribute as Advantage D3A. Considering the features that you find to be advantages of the Evrysdi tablet, how motivating are each your consideration to prescribe the Evrysdi tablet? Please use a 7-point scale, where 1=Not At All Motivating and 7=Extremely Motivating.

TABLET DISADVANTAGES

Almost half of HCPs selected the inability to take the tablet dispersion with a feeding tube as a top disadvantage of the Evrysdi Tablet.

TOP PERCEIVED EVRYSDI TABLET DISADVANTAGES

Among Total HCPs (n=100)
% Selected



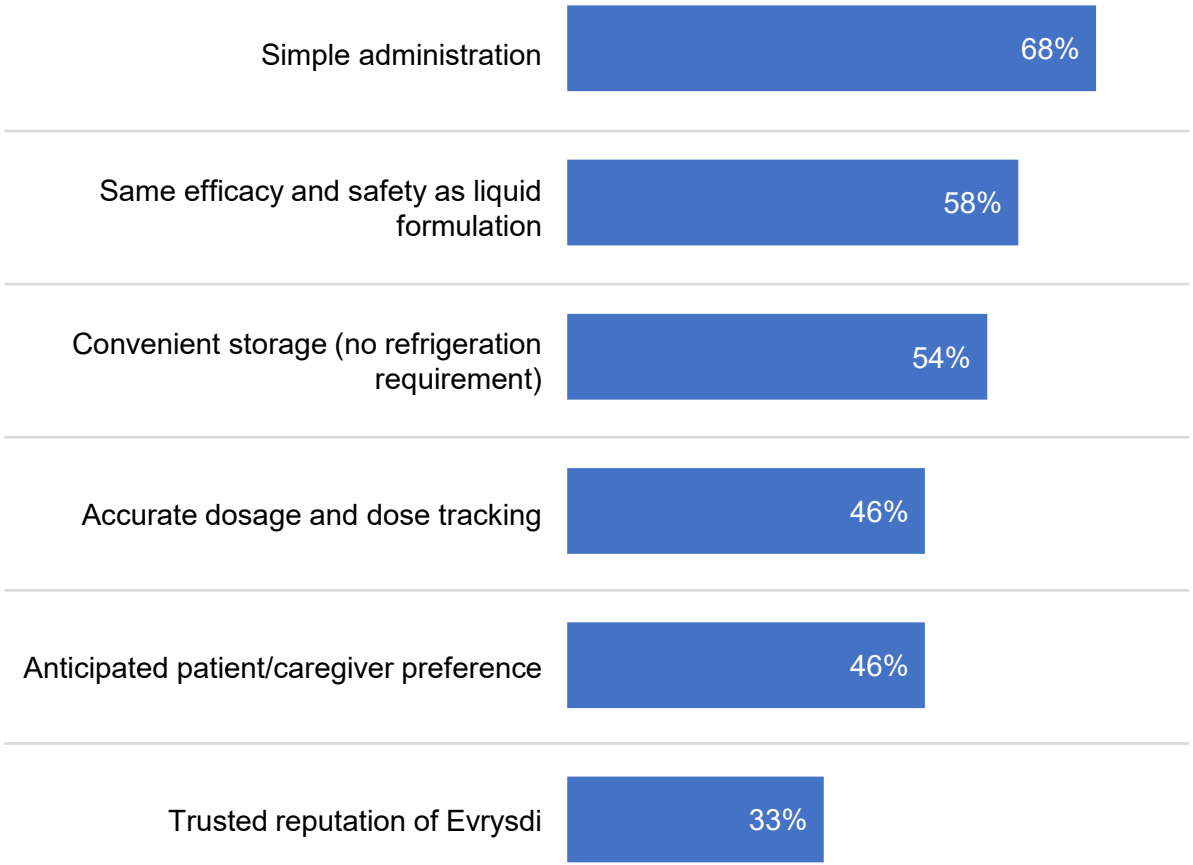
Base: Total HCPs (n=100) D3. What are the primary disadvantages of the Evrysdi Tablet for you? Please select up to 5 features in each column.

TABLET CANDIDATES AND REASONS TO SWITCH

The Evrysdi Tablet's convenient administration is a key driver of switch behavior among HCPs, who feel that the best candidates for this formulation are those who are resistant to intrathecal administration.

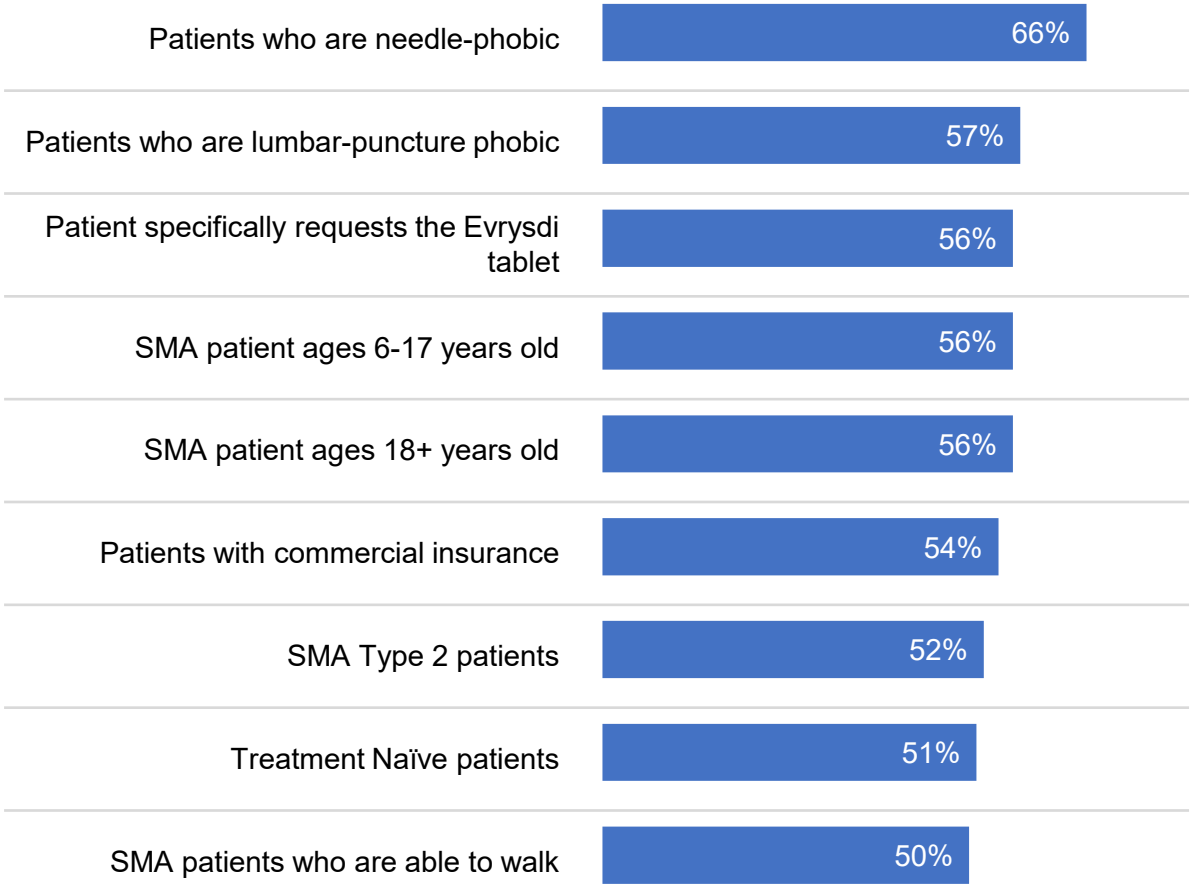
REASONS TO SWITCH/START PATIENTS ON TABLET

Among HCPs Who Would Prescribe Evrysdi Tablet (n=84)
% Selected



BEST CANDIDATES FOR EVRYSDI TABLET

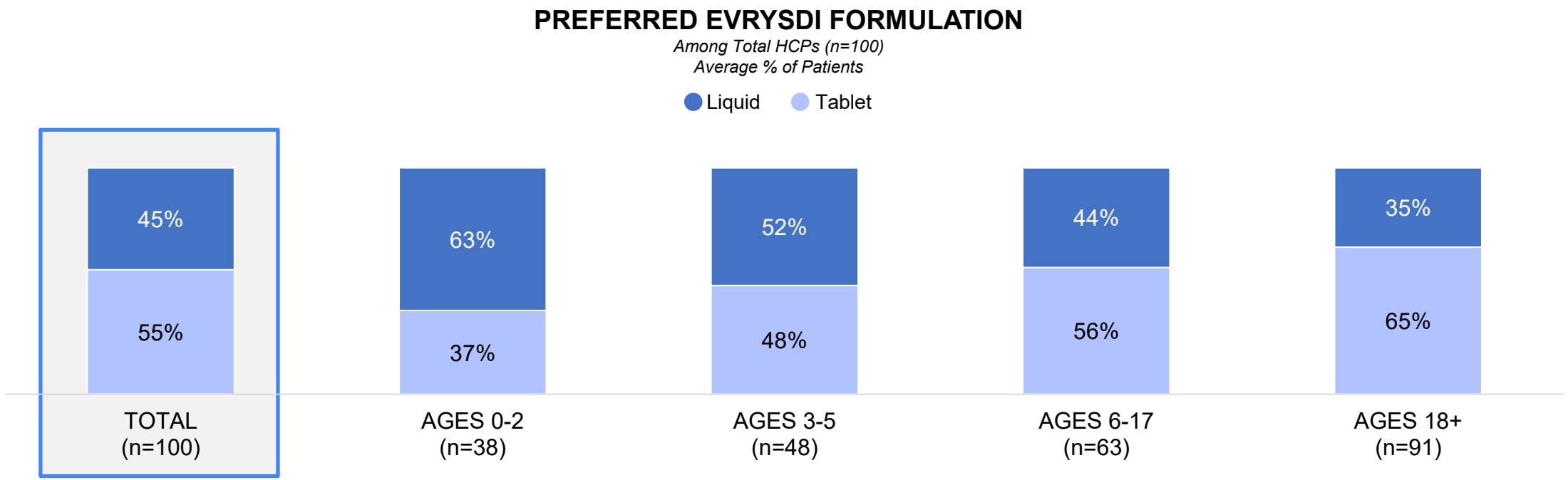
Among Total HCPs (n=100)
% Selected



Base: Would Prescribe Evrysdi Tablet (n=84) D4A. Which of the following reasons would you switch an SMA patient to the Evrysdi tablet?
Base: Total HCPs (n=100) D5. What patient types would MOST benefit from the use of the Evrysdi tablet?

PREFERRED EVRYSDI FORMULATION

HCPs indicate that preference for tablet formulation of Evrysdi increases as patient age increases.



*"The efficacy is the same, tablet is **more convenient**."*
- Pediatric Neurologist

*"They may not be old enough to reliably swallow tablets and the dispersion method **may not be family's preference**."*
– Pediatric Neurologist

*"It is **easier to use**, **easier to monitor** and has similar effect."*
- Adult Neurologist

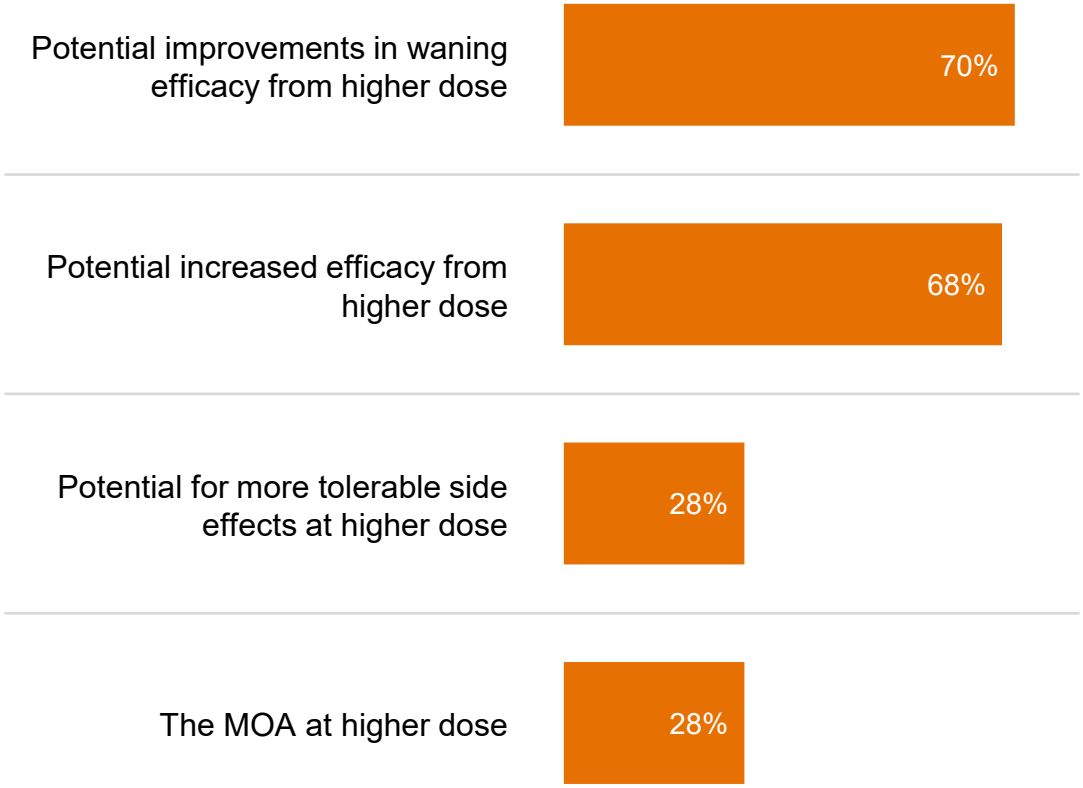
Base: Has Patients in Age Group (0-2: n=38, 3-5: n=48, 6-17: n=63, 18+: n=91) D7. For your NEXT 10 spinal muscular atrophy (SMA) patients in each of the following age groups who are Treatment Naïve (have never been treated), assuming they would all receive Evrysdi, how many would you initiate the Evrysdi Tablet vs. Evrysdi Original Formulation? / Base: Would Not Prescribe Tablet to Naïve (n=85) / D7A. Why would you NOT prescribe Evrysdi Tablet to some of your future treatment naïve SMA patients? / Base: Would Prescribe Tablet to Naive (n=99) D7B. Why would you prescribe Evrysdi Tablet to some of your future treatment naïve SMA patients?

COMPETING PRODUCTS IN DEVELOPMENT

Top motivations to prescribe High Dose Spinraza rely on an expectation for longer lasting / stronger efficacy in comparison to the current dose of Spinraza. Of those who report that they would stop patients' current DMT entirely to start them on expanded Zolgensma, most report they would need to see a decrease in function and/or efficacy in order to consider restarting patients on their current DMT.

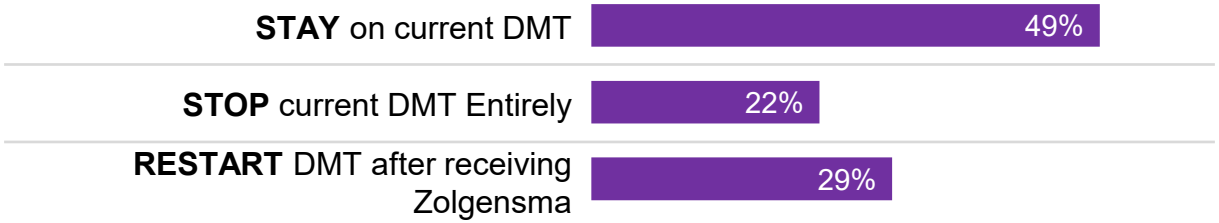
REASONS TO SWITCH/START PATIENTS ON HIGH DOSE SPINRAZA

Among HCPs Who Would Prescribe High Dose Spinraza (n=57)
% Selected



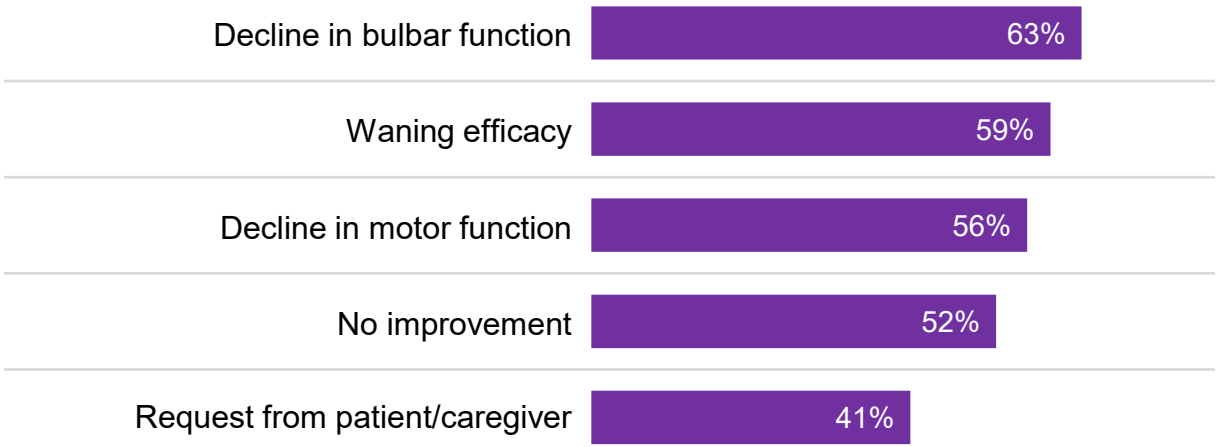
EXPECTED ZOLGENSMA TREATMENT BEHAVIOR

Among HCPs Who Would Prescribe the Expanded Indication of Zolgensma (n=35)
Average % of Patients



REASONS TO CONSIDER RESTARTING CURRENT DMT AFTER RECEIVING ZOLGENSMA

Among HCPs Who Would Stop Current DMT Entirely When Prescribing Zolgensma for 2-17 (n=27)



Base: Would Prescribe High Dose Spinraza (n=57) D10A. Which of the following reasons would you switch an SMA patient to High Dose Spinraza? / Base: Would Prescribe Expanded Indication of Zolgensma (n=35) D11A. You mentioned that you would expect some of your patients to be switched to the expanded Indication of Zolgensma to 2-17 years old in the future. Please indicate what proportion of those patients would fall under the following categories. / Base: Would Stop Patient(s) Current DMT To Initiate Zolgensma for 2-17 (n=27) D11B. Consider your patients that you would completely stop their current DMT due to switching them to the expanded Indication of Zolgensma to 2-17 years old. Which of the following reasons would be a reason for you to consider restarting a patient back on a DMT?

DETAILED FINDINGS:

PATIENTS: CURRENT TREATMENT PERCEPTIONS

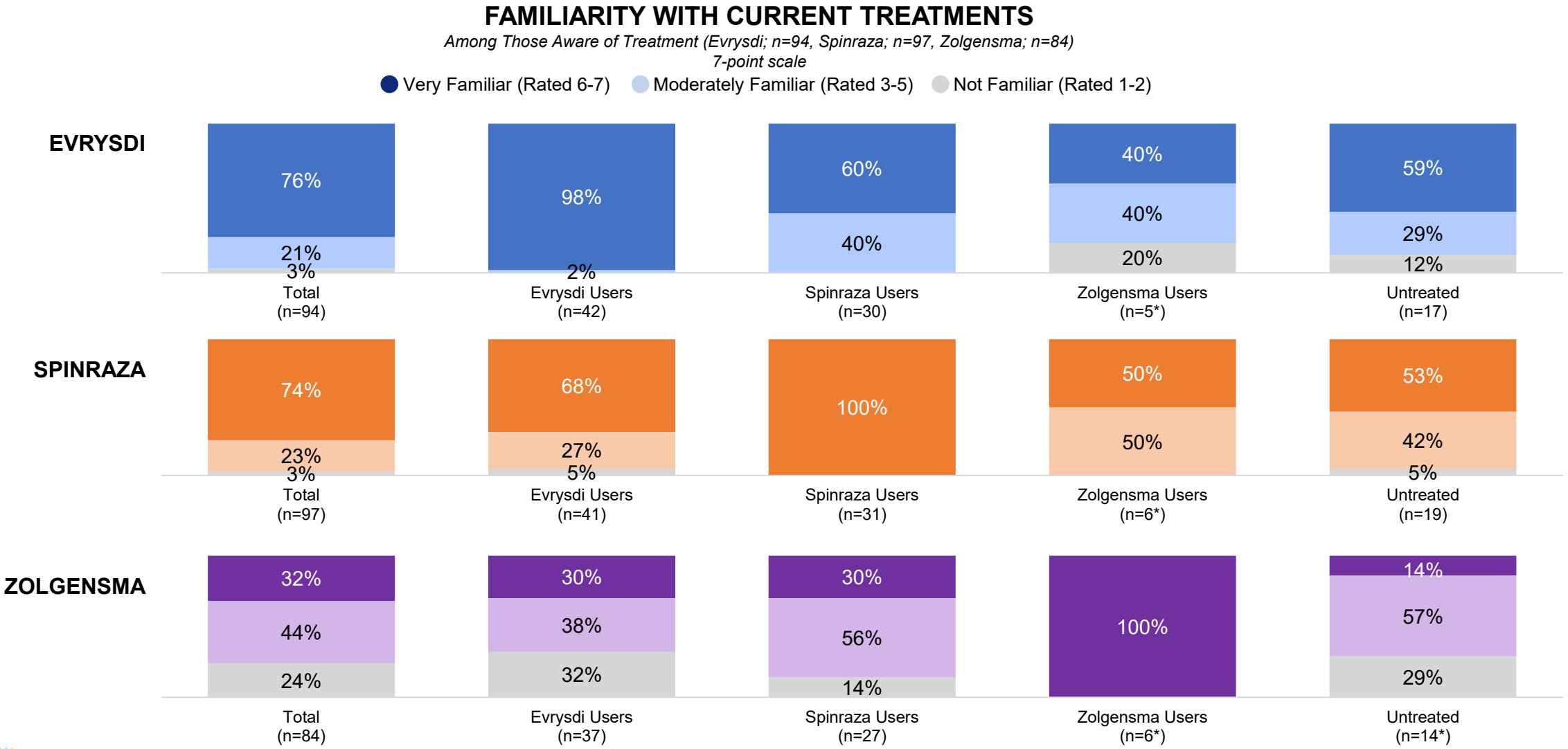
CURRENT TREATMENT PERCEPTIONS

Evrysdi and Spinraza have comparable awareness and impressions, however Evrysdi users have higher satisfaction compared to Spinraza.

Base: Total Patients (n=100) S11. Which medicines are you aware of that treat Spinal Muscular Atrophy (SMA)? / S12. Which of the following medicines have you heard of that treat SMA? / S14. For each of the following SMA treatments, please indicate which you are currently taking and have taken in the past, if any. / Base: Aware of Treatment (Evrysdi; n=94, Spinraza; n=97) A2. Using a 7-point scale where 1=Extremely Negative and 7=Extremely Positive, what is your overall impression of the following Spinal Muscular Atrophy (SMA) medicines? / Currently Taking Treatment (Evrysdi n=42, Spinraza n=31) A3. Using a 7-point scale, where 1=Not At All Satisfied and 7=Extremely Satisfied, what is your level of satisfaction with the following Spinal Muscular Atrophy (SMA) treatment that you currently use? Base: Among Currently Treated (Evrysdi Users; n=42, Spinraza Users; n=31) B5. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely are you to switch SMA medicines in the next 12 months?

CURRENT TREATMENT FAMILIARITY

Across various patient types, there is comparable familiarity with Evrysdi and Spinraza.



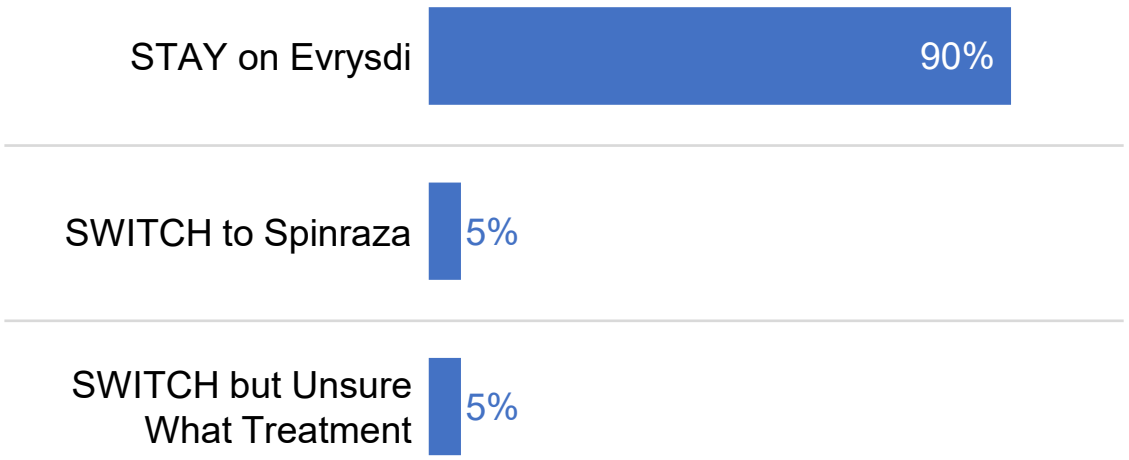
Base: Among Those Aware of Treatment (Evrysdi; n=94, Spinraza; n=97, Zolgensma; n=84)
A1. Using a 7-point scale where 1=Not At All Familiar and 7=Extremely Familiar, what is your level of familiarity with the following Spinal Muscular Atrophy (SMA) medicines?

FUTURE TREATMENT EXPECTATION WITH NO MARKET CHANGE: PATIENT

With no change in the market, the Evrysdi market share is expected to increase based on patient switching behavior. The amount of non-Evrysdi patients expected to switch to Evrysdi outweighs the number of current Evrysdi patients that expect to switch off the treatment.

EVRYSDI PATIENTS

Among Current Evrysdi Users (n=42)



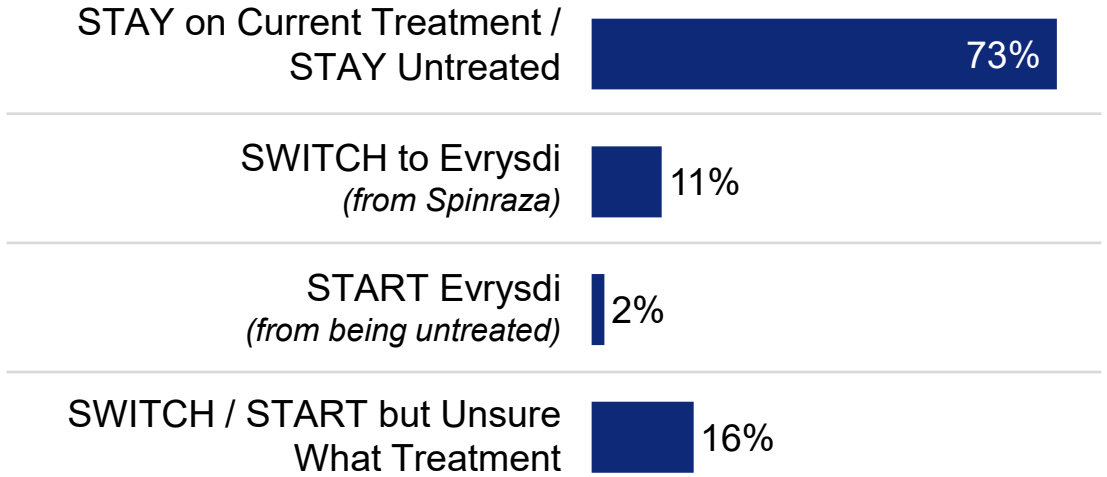
10% of current Evrysdi patients indicate that they expect to switch **OFF** Evrysdi in the next 12 months.

SHARE IMPACT:

-3.4%

NON-EVRYSDI PATIENTS

Among Naïve and Spinraza Patients (n=44)



13% of non-Evrysdi patients indicate that they expect to switch **ON** to Evrysdi in the next 12 months.

SHARE IMPACT:

+6.5%

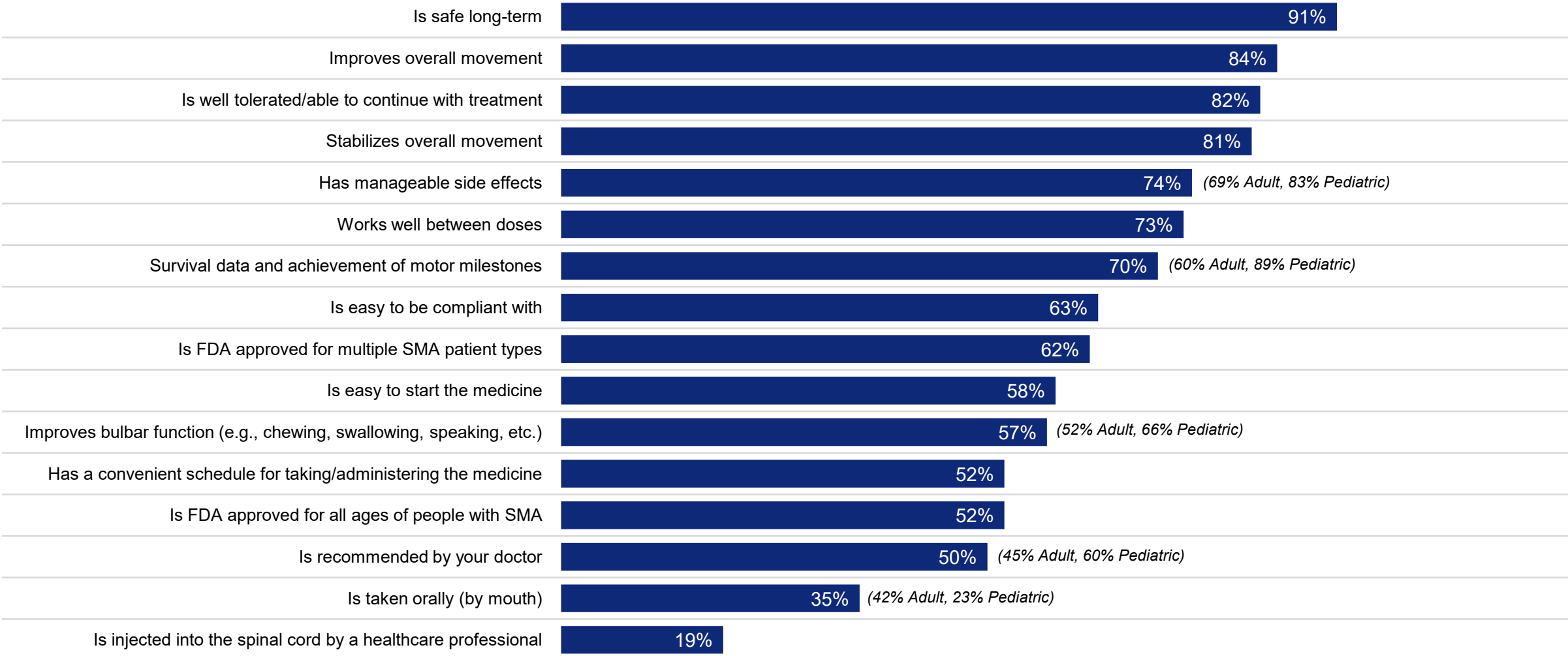
NET EVRYSDI SHARE IMPACT WITH NO CHANGES IN THE MARKET: **+3.1%** *(minus 30% deflationary adjustment)* = **+2.2%**

SMA TREATMENT ATTRIBUTES: STATED IMPORTANCE

Safety and efficacy are primary drivers for patients. Convenience factors are lower drivers.

OVERALL SMA DMT STATED USAGE DRIVERS

Among Total Patients (n=100)
7-point scale, rated 6-7



Evrysdi risdiplam

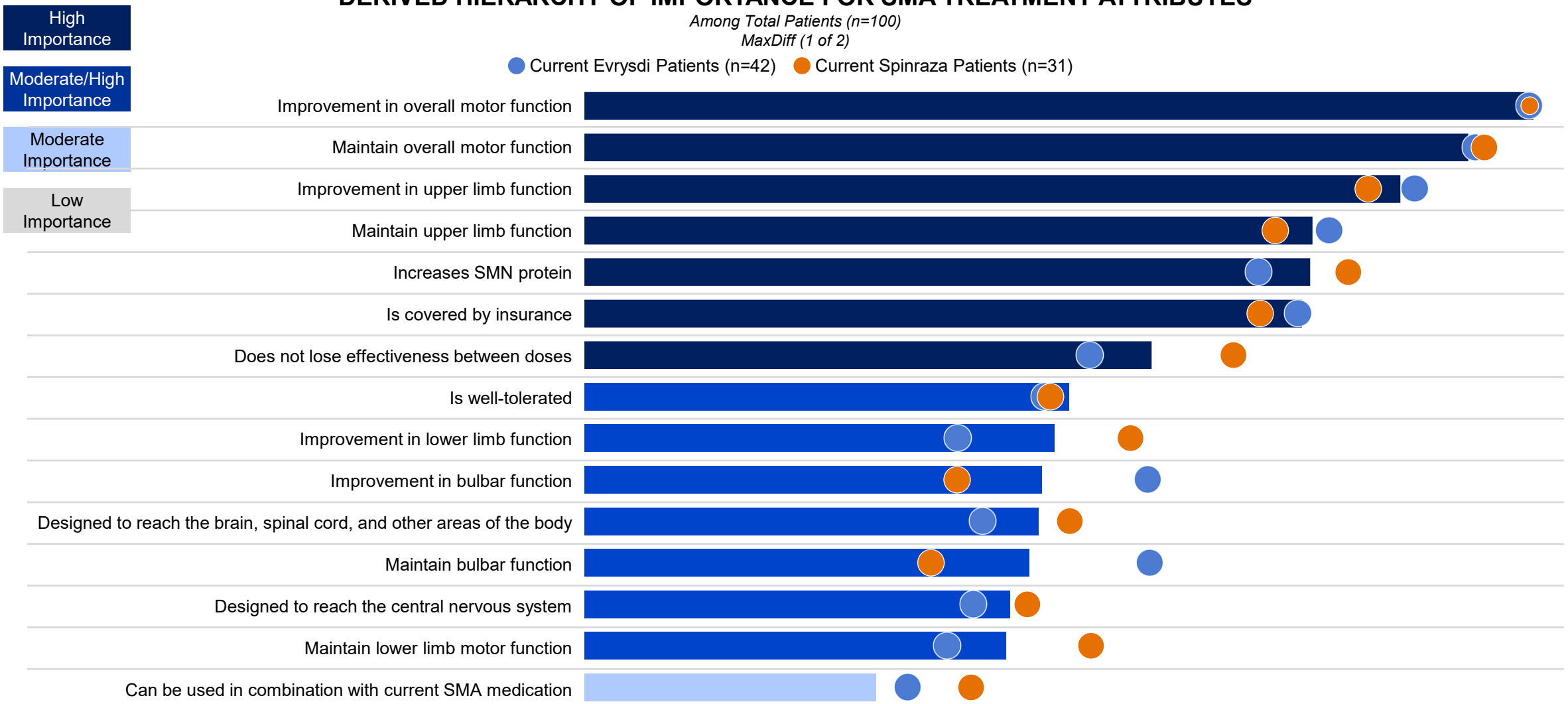
Base: Total Patients (n=100)
A4. When thinking about spinal muscular atrophy (SMA) medicines, how important is each of the following to you? Please rate the level of importance using a 7-point scale, where 1=Not At All Important and 7=Extremely Important.

SMA TREATMENT ATTRIBUTES: DERIVED IMPORTANCE

Improvement and maintaining motor function and upper limb function are top DMT drivers. Top drivers are primarily consistent among Current Evrysdi and Current Spinraza patients. Differences occur in the mid-level attributes.

DERIVED HIERARCHY OF IMPORTANCE FOR SMA TREATMENT ATTRIBUTES

Among Total Patients (n=100)
MaxDiff (1 of 2)



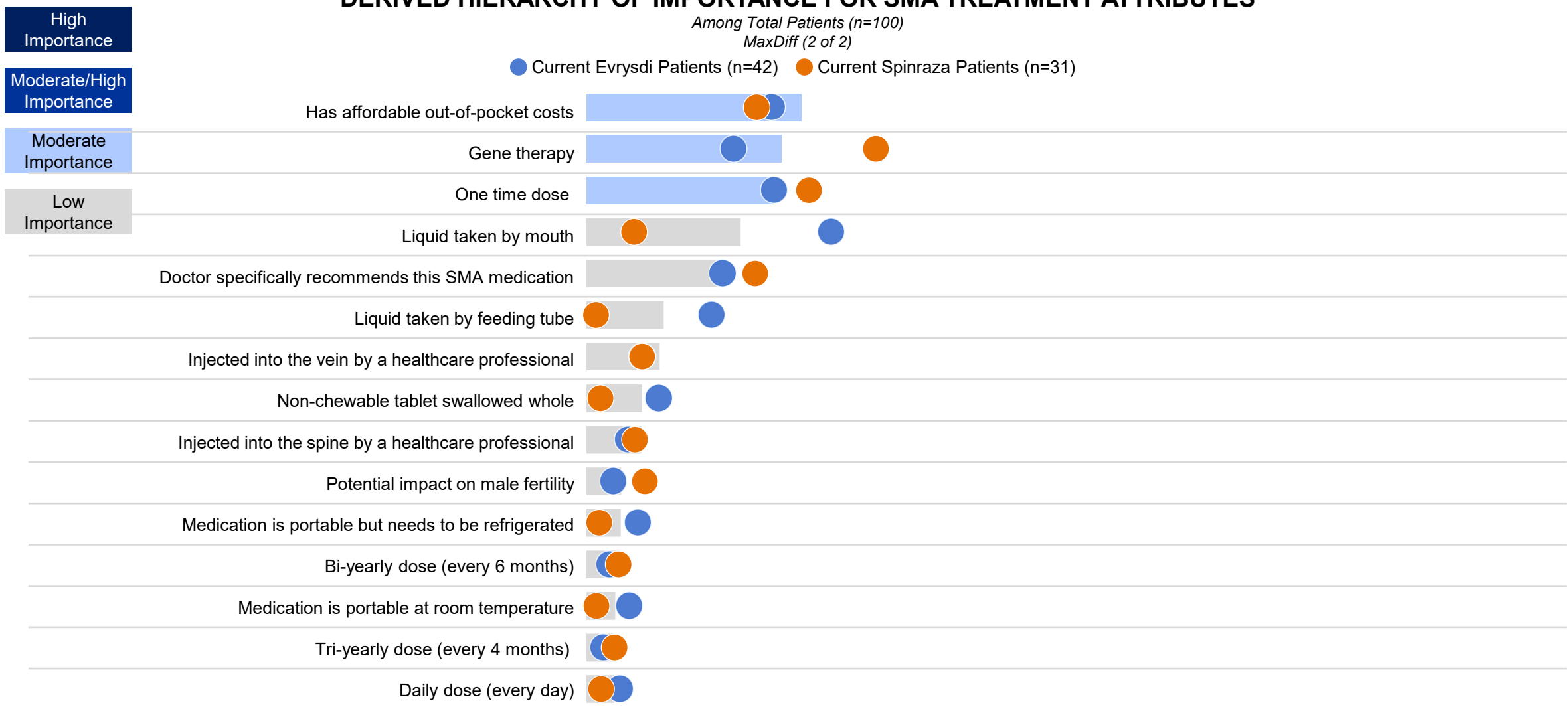
Base: Total Patients (n=100) E1. When thinking about new spinal muscular atrophy (SMA) medicines, we would like to understand the importance of various product features. On each screen, please select which SMA product feature would be MOST important to you and which SMA product feature would be LEAST important to you.

SMA TREATMENT ATTRIBUTES DERIVED IMPORTANCE

Less important drivers are primarily consistent among Current Evrysdi and Current Spinraza patients.

DERIVED HIERARCHY OF IMPORTANCE FOR SMA TREATMENT ATTRIBUTES

Among Total Patients (n=100)
MaxDiff (2 of 2)



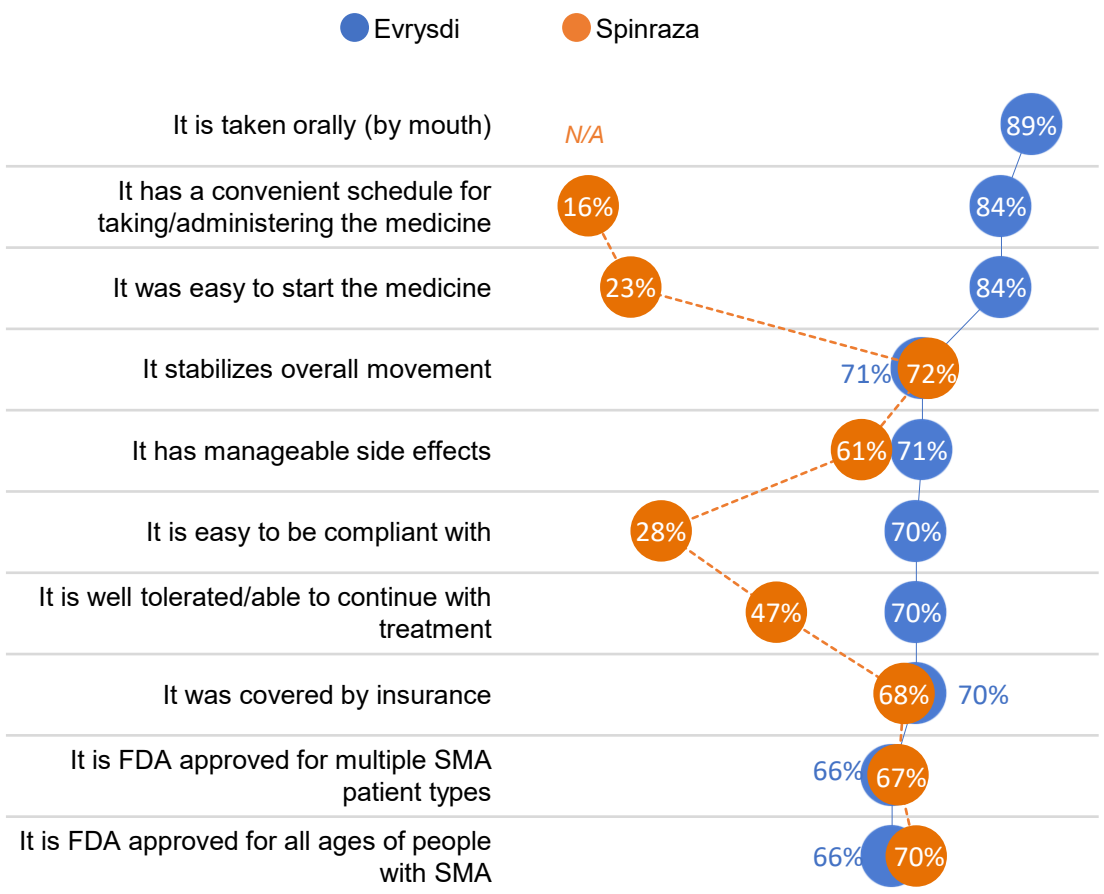
Base: Total Patients (n=100) E1. When thinking about new spinal muscular atrophy (SMA) medicines, we would like to understand the importance of various product features. On each screen, please select which SMA product feature would be MOST important to you and which SMA product feature would be LEAST important to you.

USAGE DRIVERS: EVRYSDI AND SPINRAZA

Convenience is primary driver for Evrysdi, whereas Spinraza is due to lack of options being available and that it was recommended by their doctor and covered by insurance, in addition to efficacy.

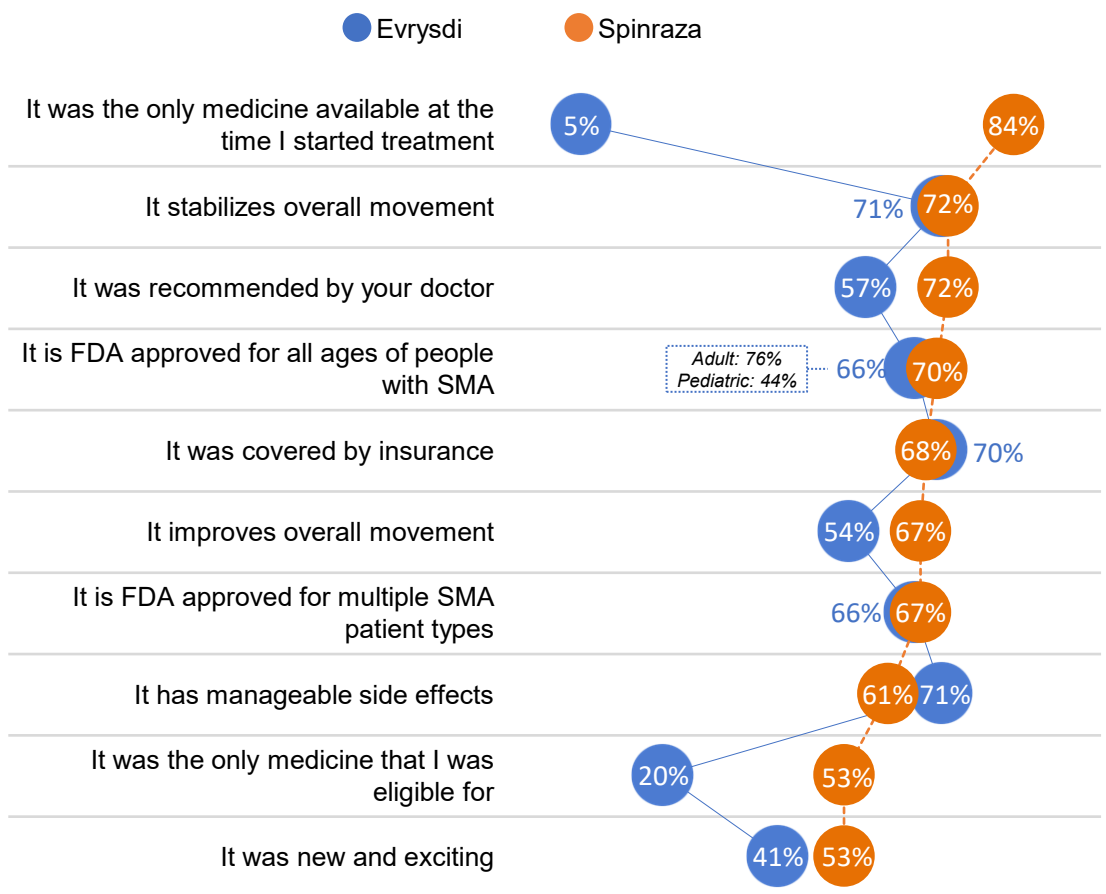
TOP REASONS TO USE EVRYSDI

Spinraza Data Shown for Comparison
Current or Past User (Evrysdi: n=56, Spinraza: n=57)



TOP REASONS TO USE SPINRAZA

Evrysdi Data Shown for Comparison
Current or Past User (Evrysdi: n=56, Spinraza: n=57)



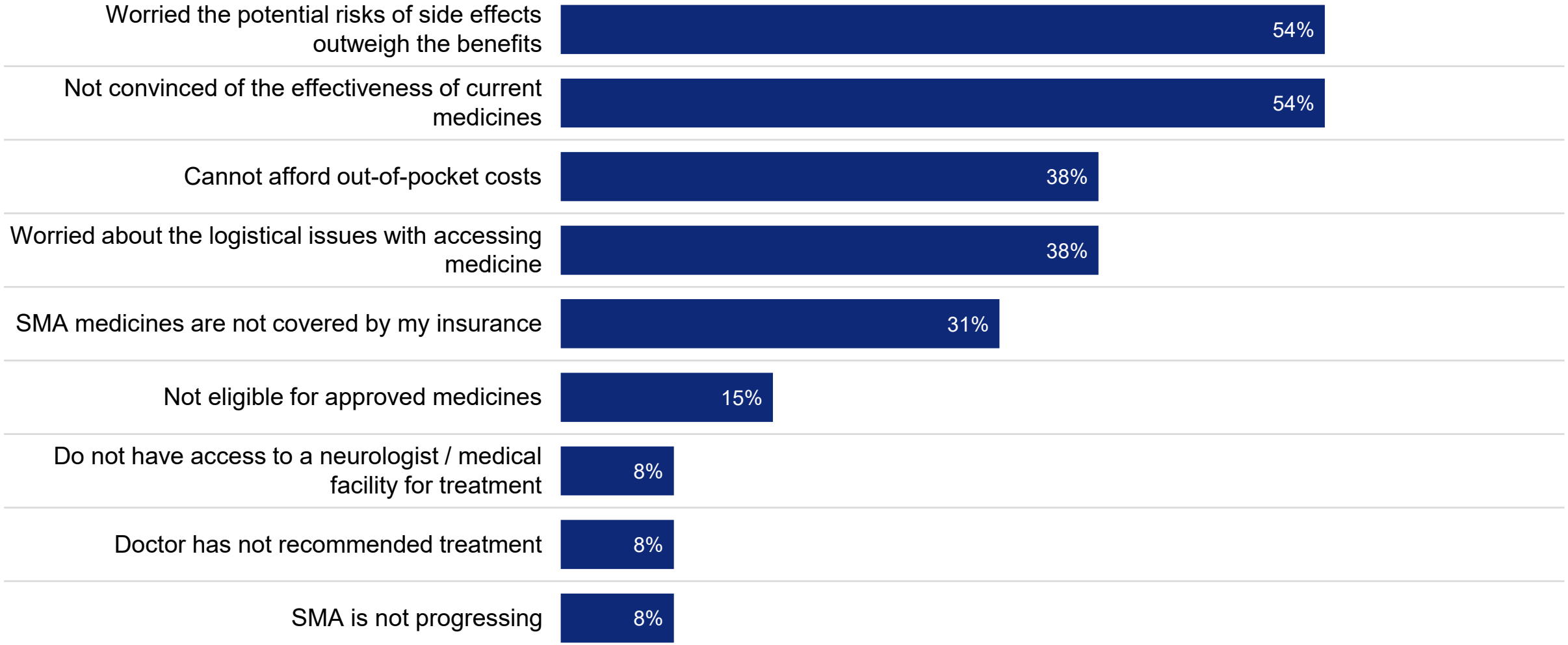
Base: Current or Past User (Evrysdi: n=56, Spinraza: n=57)
B1. For which of the following reasons did you choose to use the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?
*Full list can be found in the Patient appendix

USAGE BARRIERS: UNTREATED

Untreated patients hold concerns about the efficacy and safety of current treatments.

TREATMENT NAÏVE BARRIERS TO TREATMENT

Treatment Naïve Patients (n=13)*
% Selected



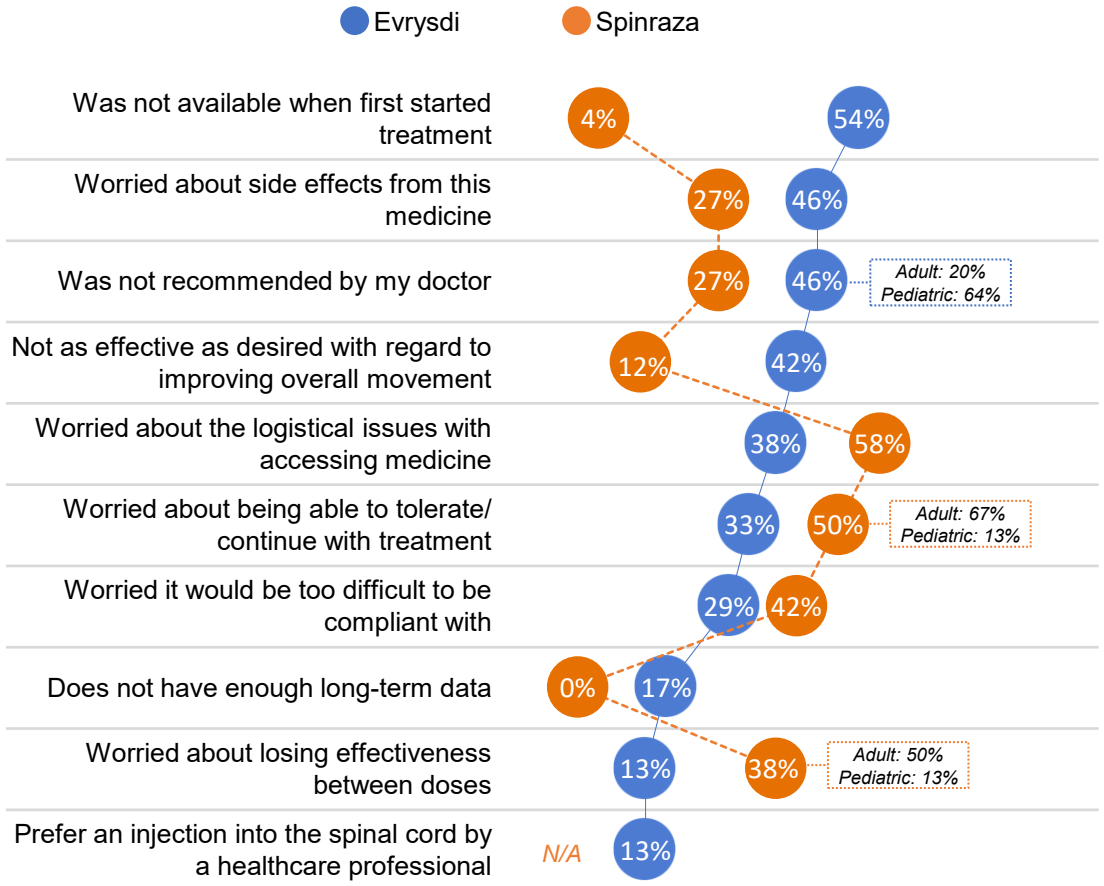
*CAUTION: SMALL BASE
Base: Treatment Naïve (n=13*)
B8. For which of the following reasons have you never used any medications for the treatment of spinal muscular atrophy (SMA)?

USAGE BARRIERS: EVRYSDI AND SPINRAZA

Many who have never used Evrysdi credit its unavailability when starting treatment, concerns about safety and efficacy, and it not recommended by their doctor for the reasons why. Many indicate a preference towards oral medication as well as logistical concerns and not being easy to start as reasons for never using Spinraza.

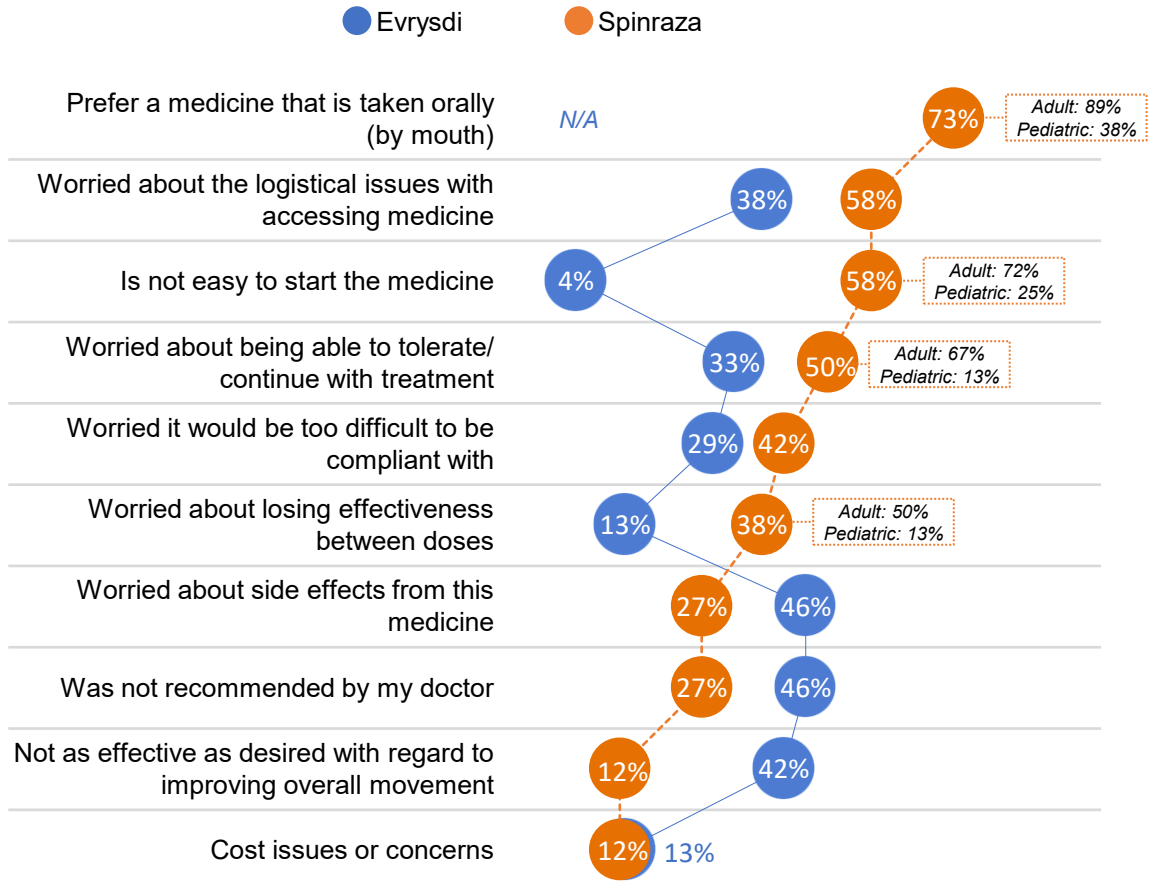
TOP REASONS FOR NEVER USING EVRYSDI

Spinraza Data Shown for Comparison
Currently or Previously Treated but Never Treated with Evrysdi (n=24) or Spinraza (n=26)



TOP REASONS FOR NEVER USING SPINRAZA

Evrysdi Data Shown for Comparison
Currently or Previously Treated but Never Treated with Evrysdi (n=24) or Spinraza (n=26)



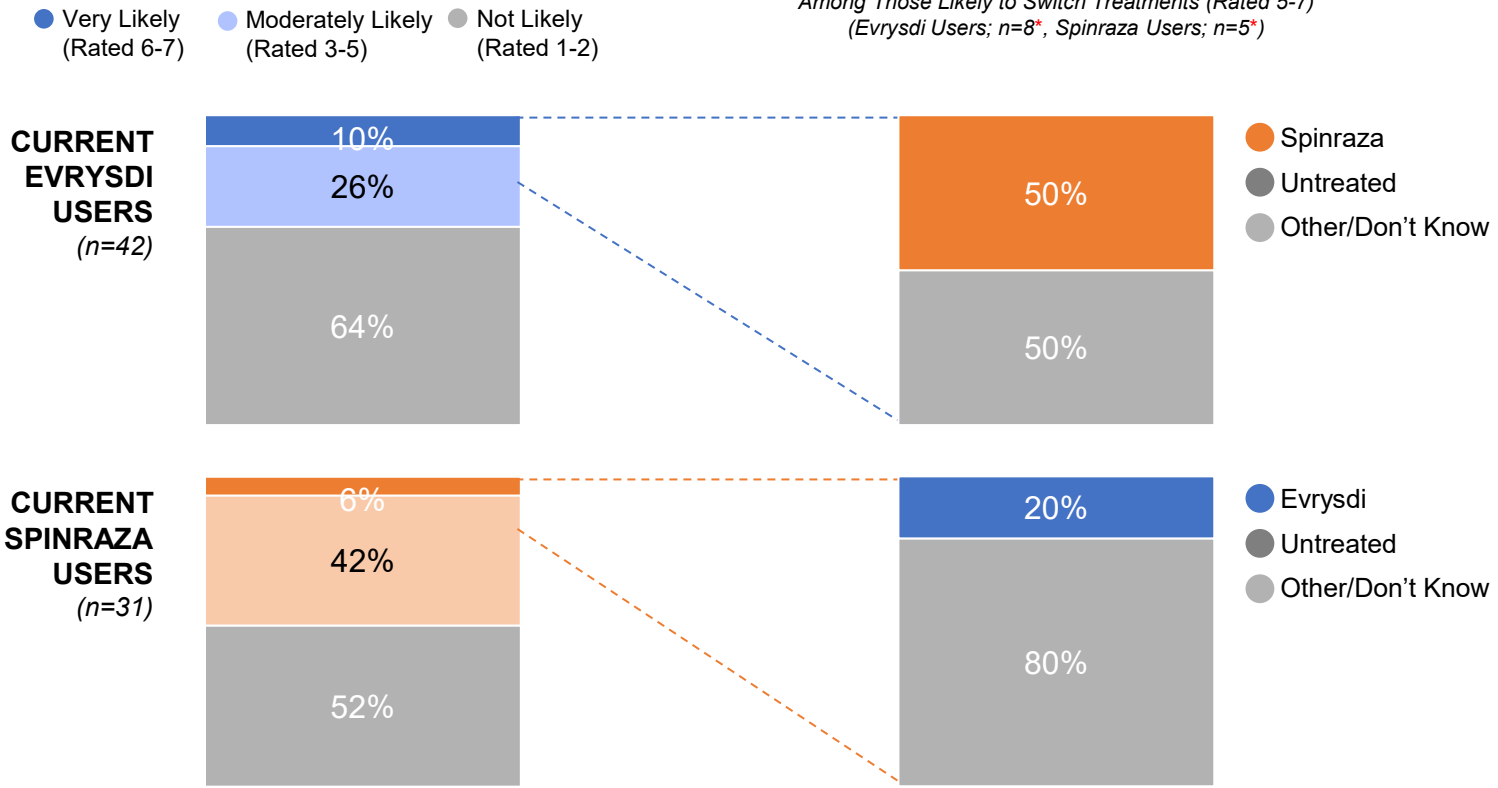
Base: Currently or Previously Treated but Never Treated with Evrysdi (n=24) or Spinraza (n=26)
B2. For which of the following reasons have you never used the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?
*Full list can be found in the Patient appendix

PATIENT SWITCH BEHAVIOR: CURRENTLY TREATED

Only a few are very likely to switch, however there are some that are undecided and may change their likelihood in the next 12 months. Fence sitters can make an impact on future usage.

LIKELIHOOD TO SWITCH DMT

7-point scale
Among Currently Treated Patients



DISCUSSION

Among Likely to Switch (Evrysdi Users; n=8*, Spinraza Users; n=5*)

*"Unfortunately, each time [on Evrysdi] I begin to diminish considerably after the 6-month mark, but I would **still switch back** to it in order to experience the 6 months of increased strength."*
- Adult Patient, Current Spinraza User

*"I feel that I had **better improvements from Spinraza**. I am also interested in the possible higher dose of Spinraza getting FDA approved."*
– Pediatric Caregiver, Current Evrysdi User

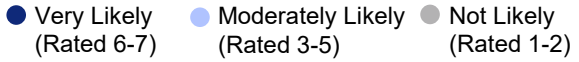
*CAUTION: SMALL BASE
Base: Among Currently Treated (Evrysdi Users; n=42, Spinraza Users; n=31) B5. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely are you to switch SMA medicines in the next 12 months? / Base: Likely to Switch Treatments (Evrysdi Users; n=8*, Spinraza Users; n=5*) B6. Which SMA medicine would you be most likely to switch to in the next 12 months? / B7A./B7B./B7C. Why would you switch to Evrysdi (risdiplam) / Spinraza (nusinersen) / Zolgensma (onasemnogene aberparvovec)?

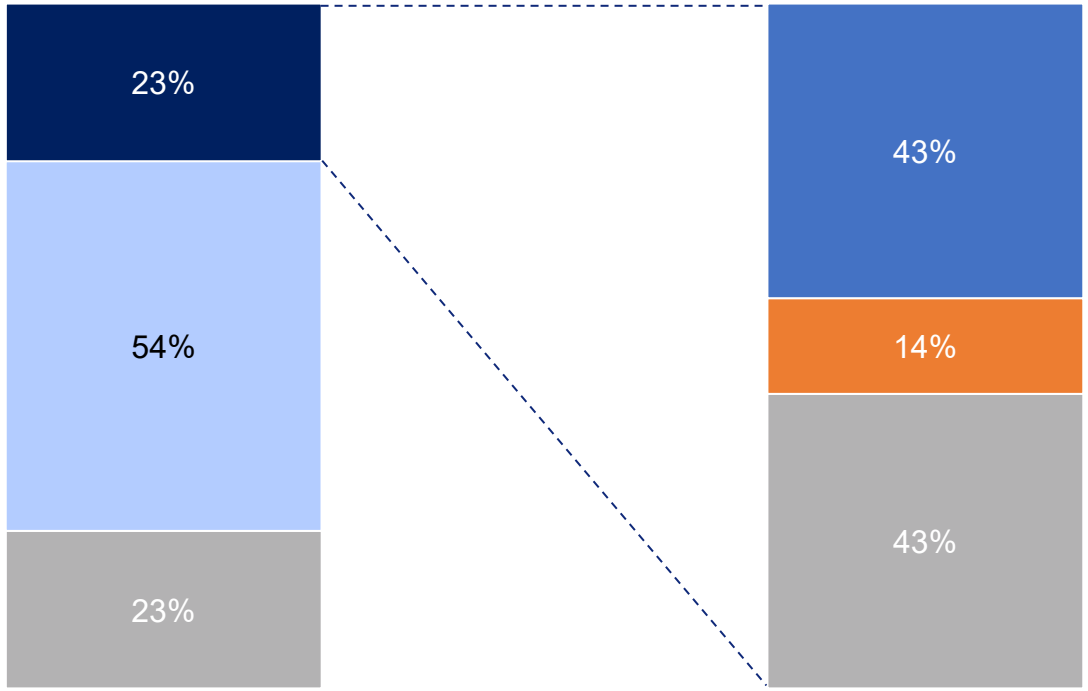
PATIENT SWITCH BEHAVIOR: CURRENTLY TREATED

For the Treatment Naïve patients, there is interest to start on a DMT and many anticipate starting on Evrysdi.

LIKELIHOOD TO START DMT

Among Treatment Naïve (n=13)*
7-point scale





ANTICIPATED TREATMENT TO START

Among Treatment Naïve Likely to Start Treatment (n=7)*

DISCUSSION

Among Treatment Naïve Likely to Start DMT (n=7)*

*"It's **easy to take** medication orally."*
– Adult Patient, Treatment Naive

*"I've **heard more** about [Spinraza] than the [Evrysdi]."*
– Pediatric Caregiver, Treatment Naive

*"It is an **oral** medication and not a shot in my spine. Very worried by getting shots in my spine even if they are very effective."*
– Adult Patient, Treatment Naive

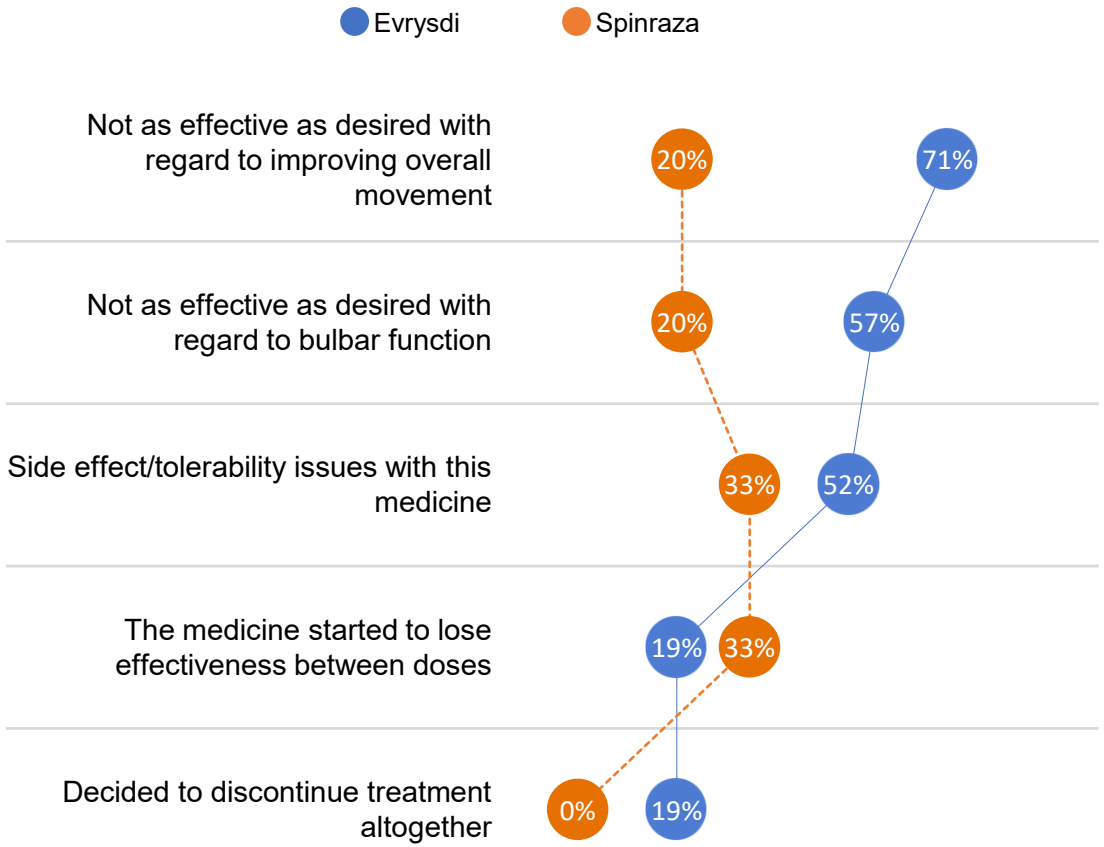
*CAUTION: SMALL BASE
Base: Treatment Naïve (n=13*) B9. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely are you to take a SMA medicine in the next 12 months?
Base: Among Treatment Naïve Likely to Start DMT (n=7*) B10. Which SMA medicine would you most likely take in the next 12 months?
B11A./B11B/B11C. Why would you take Evrysdi (risdiplam) / Spinraza (nusinersen) / Zolgensma (onasemnogene aberparvovec)?

DISCONTINUATIONS: EVRYSDI AND SPINRAZA

For those previously treated, efficacy is a primary reason to discontinue Evrysdi, whereas its burdensome administration for Spinraza.

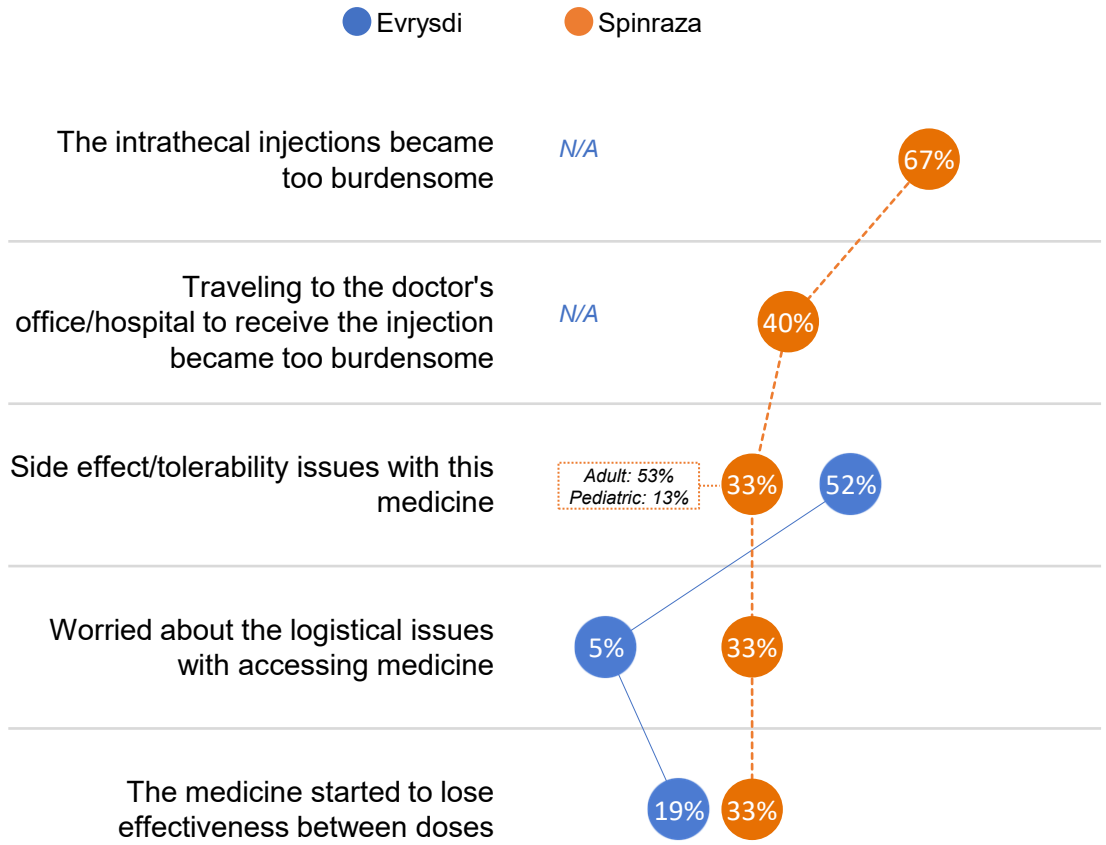
TOP REASONS FOR DISCONTINUING EVRYSDI

Spinraza Data Shown for Comparison
Previously Treated with Evrysdi, but not Currently (n=21)



TOP REASONS FOR DISCONTINUING SPINRAZA

Evrysdi Data Shown for Comparison
Previously Treated with Spinraza, but not Currently (n=30)



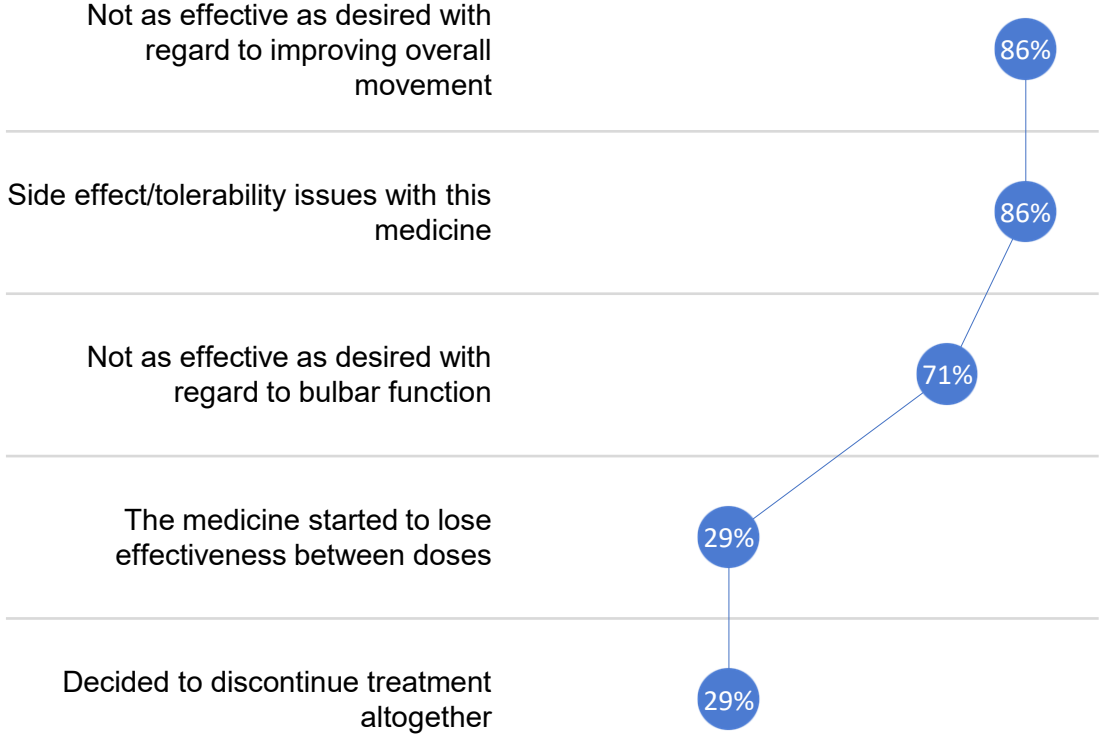
Base: Currently or Previously Treated (Evrysdi Current/Past Users; n=21, Spinraza Current/Past Users; n=30)
C2. For which of the following reasons did you stop using the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?
*Full list can be found in the Patient appendix

DISCONTINUATION AND STOP: EVRYSDI AND SPINRAZA

For those who discontinued Evrysdi and are currently untreated, side effects jump to a top reason for discontinuing along with efficacy concerns. All of hose who discontinued Spinraza and are currently untreated cite a lack of efficacy regarding improving overall movement as a reason for discontinuing.

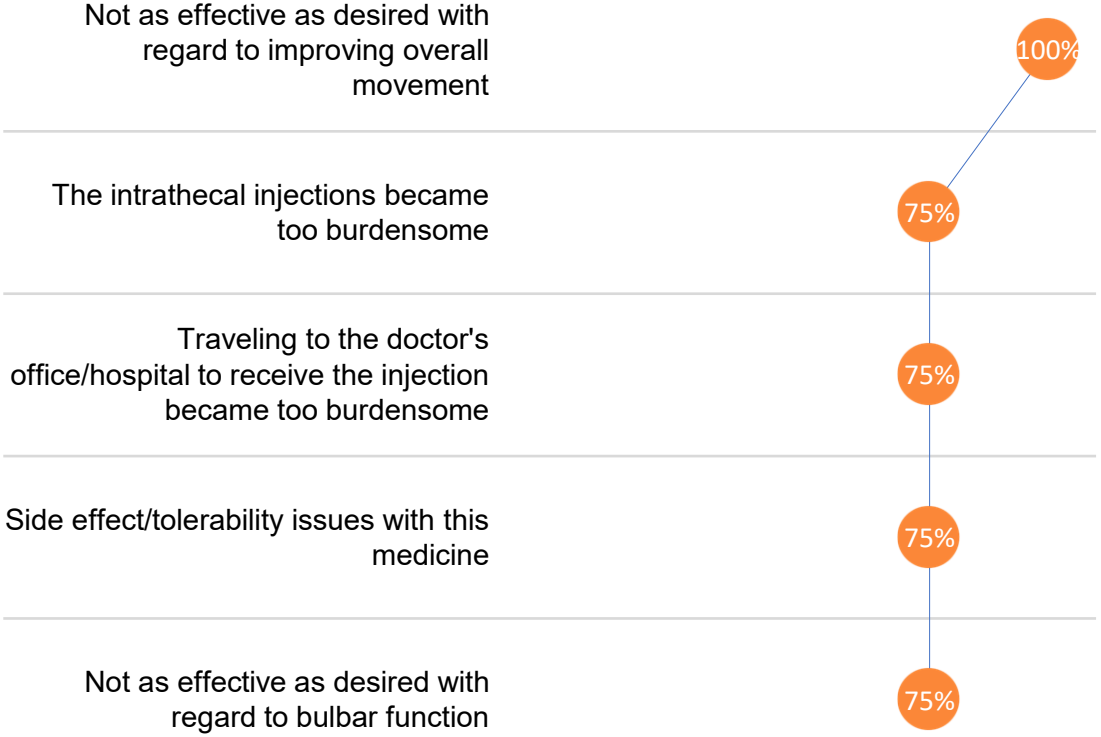
TOP REASONS FOR DISCONTINUING EVRYSDI

Discontinued Evrysdi and Not Currently Treated (n=7)*



TOP REASONS FOR DISCONTINUING SPINRAZA

Discontinued Spinraza and Not Currently Treated (n=4)*



*CAUTION: SMALL BASE

Base: Discontinued Treatment and Not Currently Treated (Evrysdi: n=7*, Spinraza: n=4*)

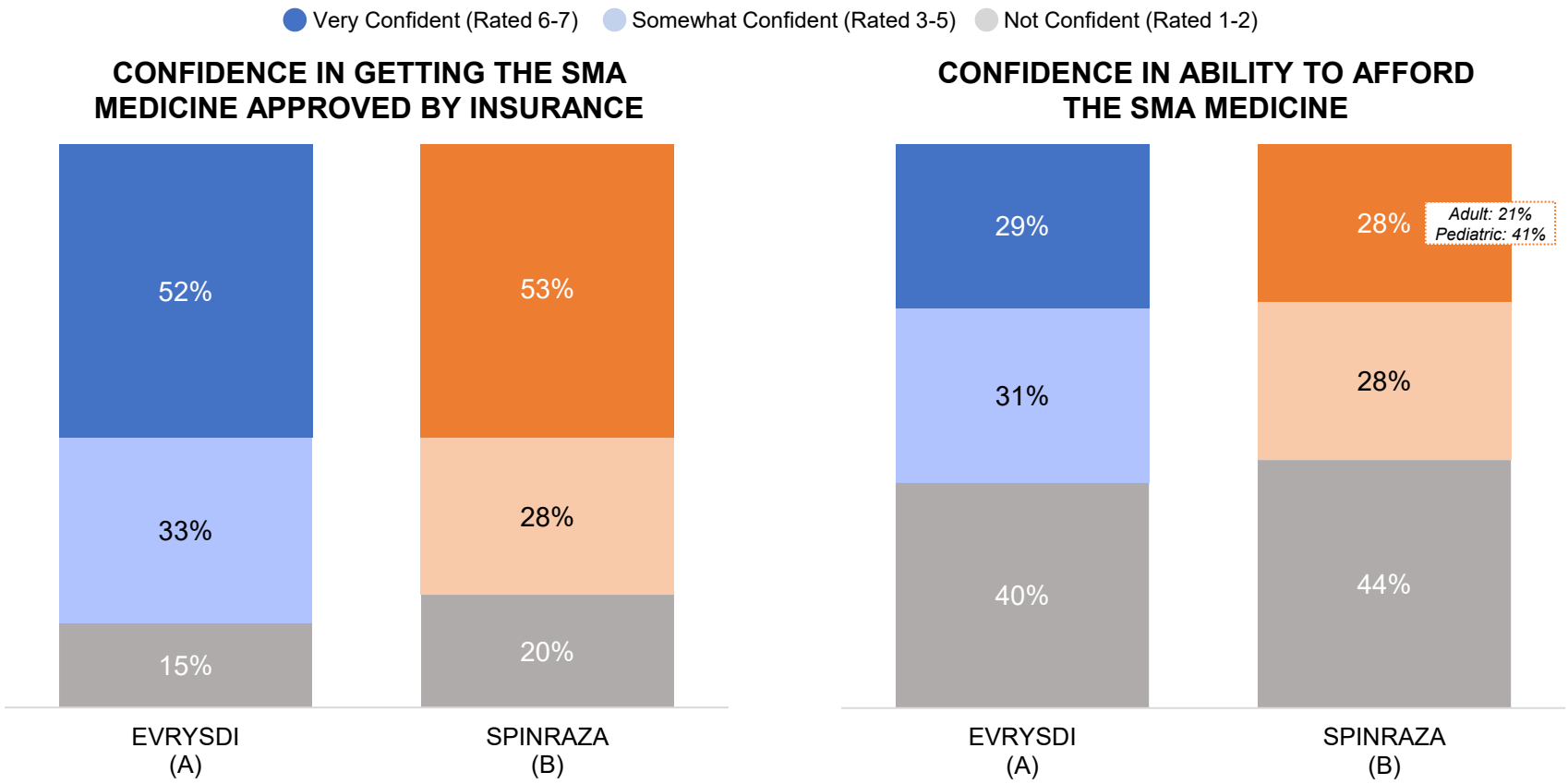
C2. For which of the following reasons did you stop using the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

ACCESS & AFFORDABILITY

Majority were confident on the insurance approval, but many had lower confidence in between able to afford the SMA medicine. Almost half were denied with similar denial rates for both Evrysdi and Spinraza.

PATIENT CONFIDENCE IN ACCESS AND AFFORDABILITY

Aware of Medication (Evrysdi: n=94, Spinraza: n=97)
7-point scale



OVERALL DENIAL RATE

Total (n=100)

46%
Received Denial For At Least One SMA Treatment

TREATMENT DENIED BY INSURANCE

Among Those Denied SMA Treatment (n=46)

57%
Received Evrysdi Denial

50%
Received Spinraza Denial

11%
Received Zolgensma Denial

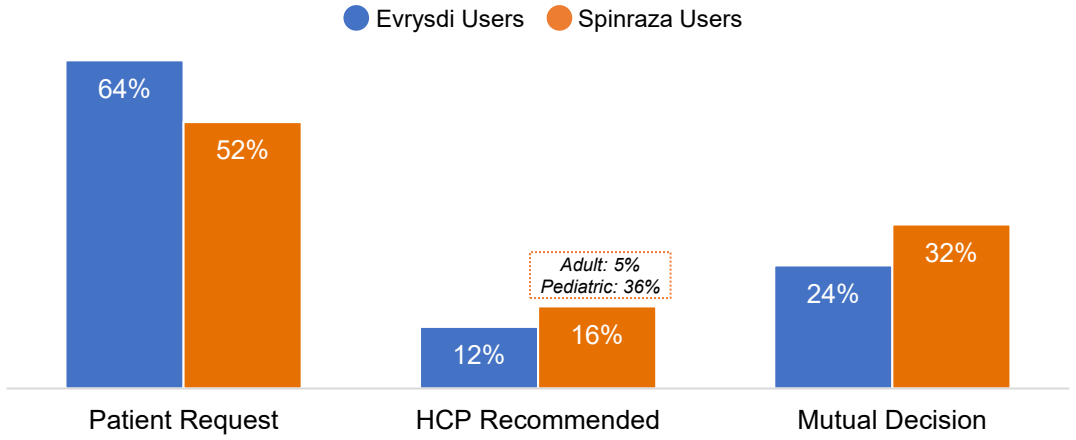
Base: Aware of Medication (Evrysdi: n=94, Spinraza: n=97) C3. Using a 7-point scale, where 1=Not At All Confident and 7=Extremely Confident, how confident are you in receiving insurance approvals and affordability of the following SMA medicines? / Base: Total (n=100) C4. Has your insurance company ever denied you access to an SMA medicine? / Base: Denied Access to SMA Medicine (n=46) C5. Which medicine(s) did your insurance company deny access to?

HEALTHCARE ENGAGEMENT

Patients have a significant impact on treatment decisions, with most making a treatment decision based on a request or a mutual decision with their HCP. If an HCP makes a recommendation, more than two-third of patients accept the recommendation on average, with caregivers of pediatric patients relying more heavily on their HCP recommendation.

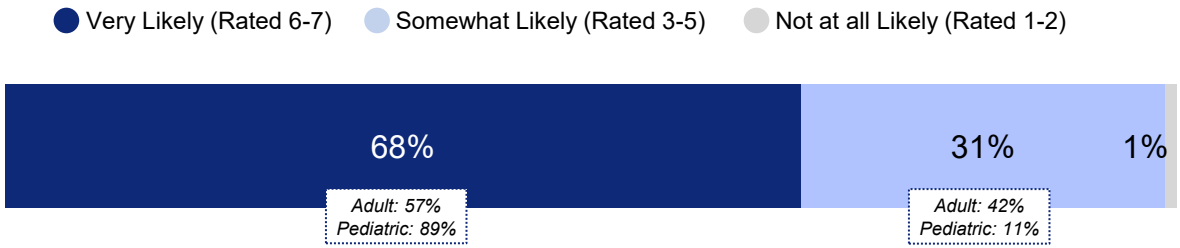
TREATMENT DECISIONS

Among Current Users (Evrysdi: n=42, Spinraza: n=31)



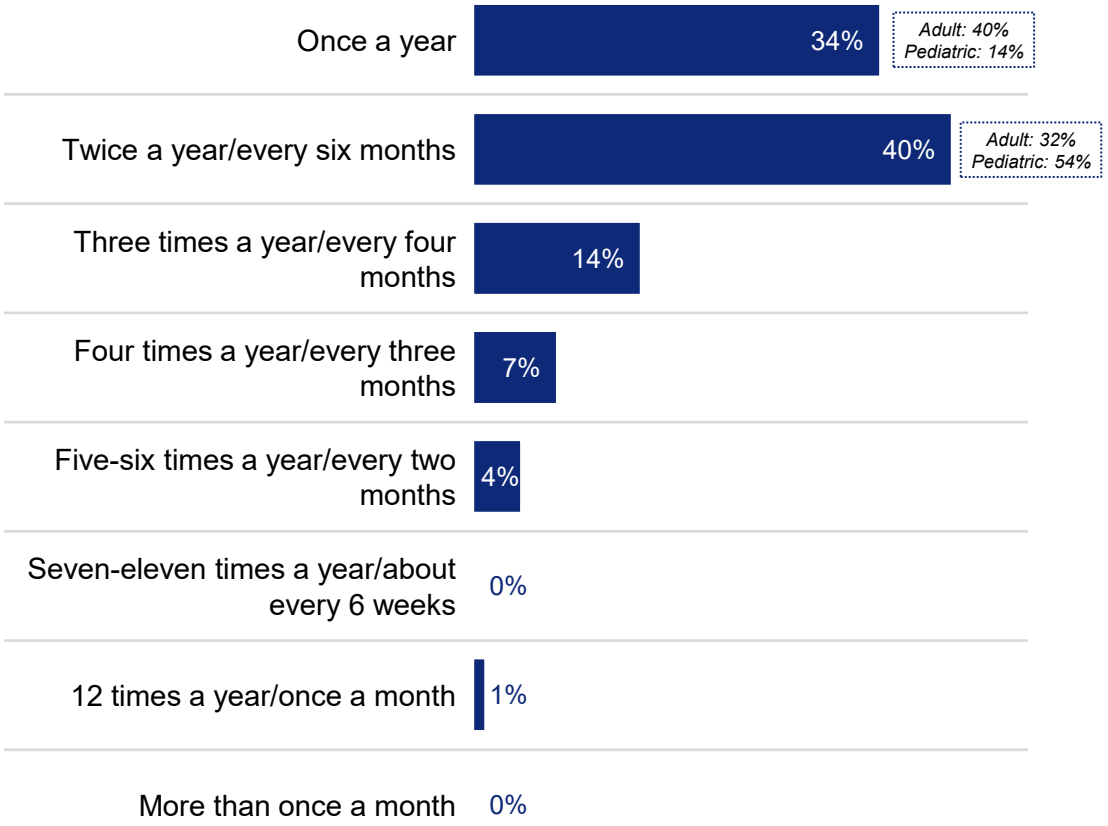
LIKELIHOOD TO ACCEPT HCP TREATMENT RECOMMENDATION

Among Total (n=100)
7-point scale



FREQUENCY OF NEUROLOGIST VISITS

Among Total (n=100)



Base: Currently Treated (Evrysdi: n=42, Spinraza; n=31) B3. When choosing the following medicine(s), did you specifically request the medicine from your doctor or was your doctor the first to recommend it?
Base: Total Patients (n=100) B4. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely are you to accept the spinal muscular atrophy (SMA) medicine your doctor recommends? / B12. In a typical year, how often do you see the doctor who primarily treats your spinal muscular atrophy (SMA)?

DETAILED FINDINGS:

PATIENTS: TREATMENTS IN DEVELOPMENT

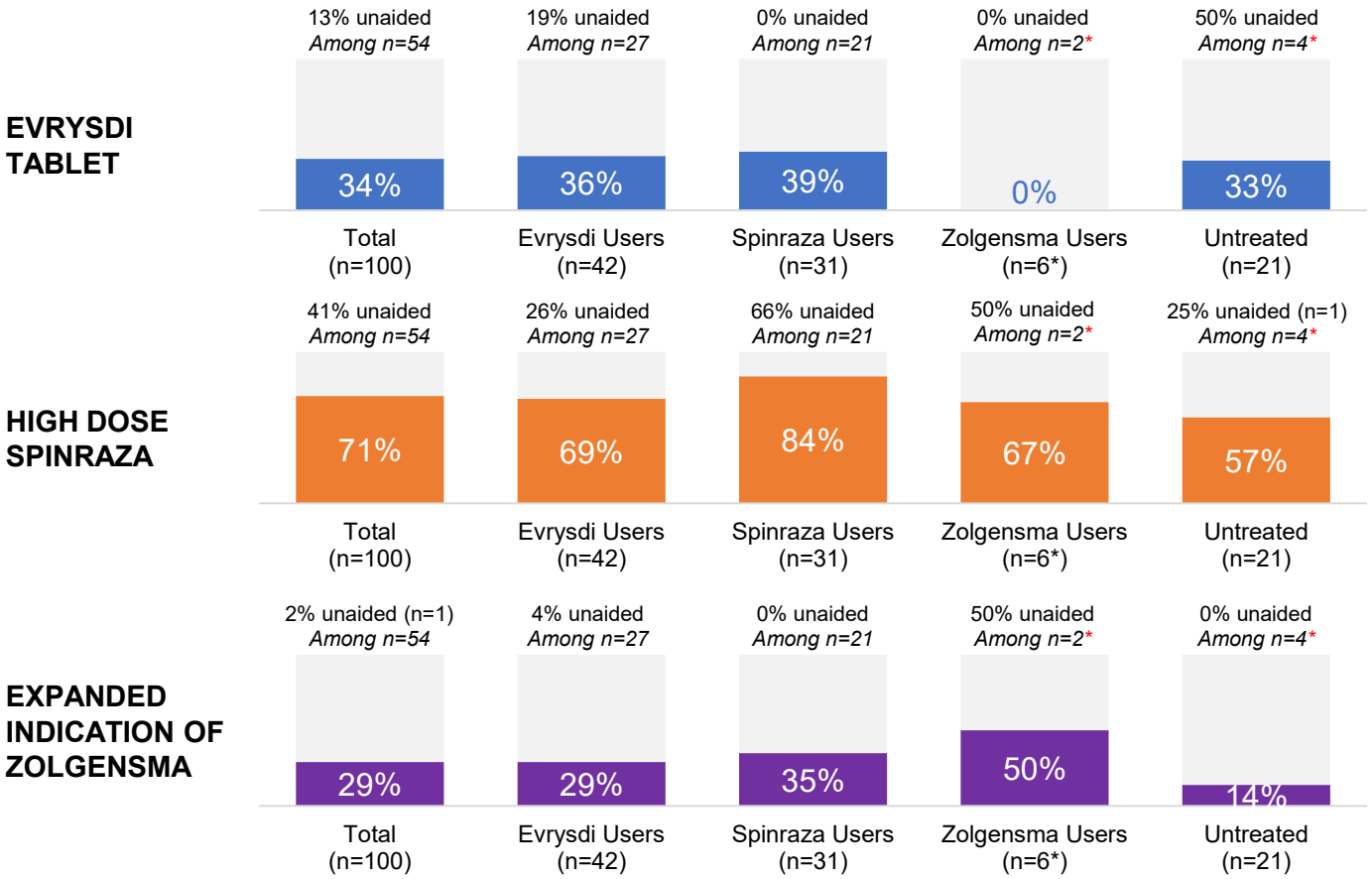
Evrysdi® risdiplam 60 mg powder for oral solution

AWARENESS OF TREATMENTS IN DEVELOPMENT

Patients have moderate awareness of the Evrysdi Tablet and the expanded indication of Zolgensma, while a majority are aware of High Dose Spinraza.

AWARENESS OF TREATMENTS IN DEVELOPMENT

Among Total (n=100)



DISCUSSION

Among Those Aware of Treatment in Development

*"It could make it **easier to travel with.** It doesn't have to be refrigerated."*
– Pediatric Caregiver, Current Evrysdi User

*"It may be more effective for adults and **might help with the dip in strength** that occurs when the drug is wearing off at the end of the third month after treatment."*
– Adult Patient, Current Spinraza User

*"An **intrathecal administration is being developed for older, larger children** rather than the intravenous for the smaller, younger children."*
– Pediatric Caregiver, Current Evrysdi User

*CAUTION: SMALL BASE

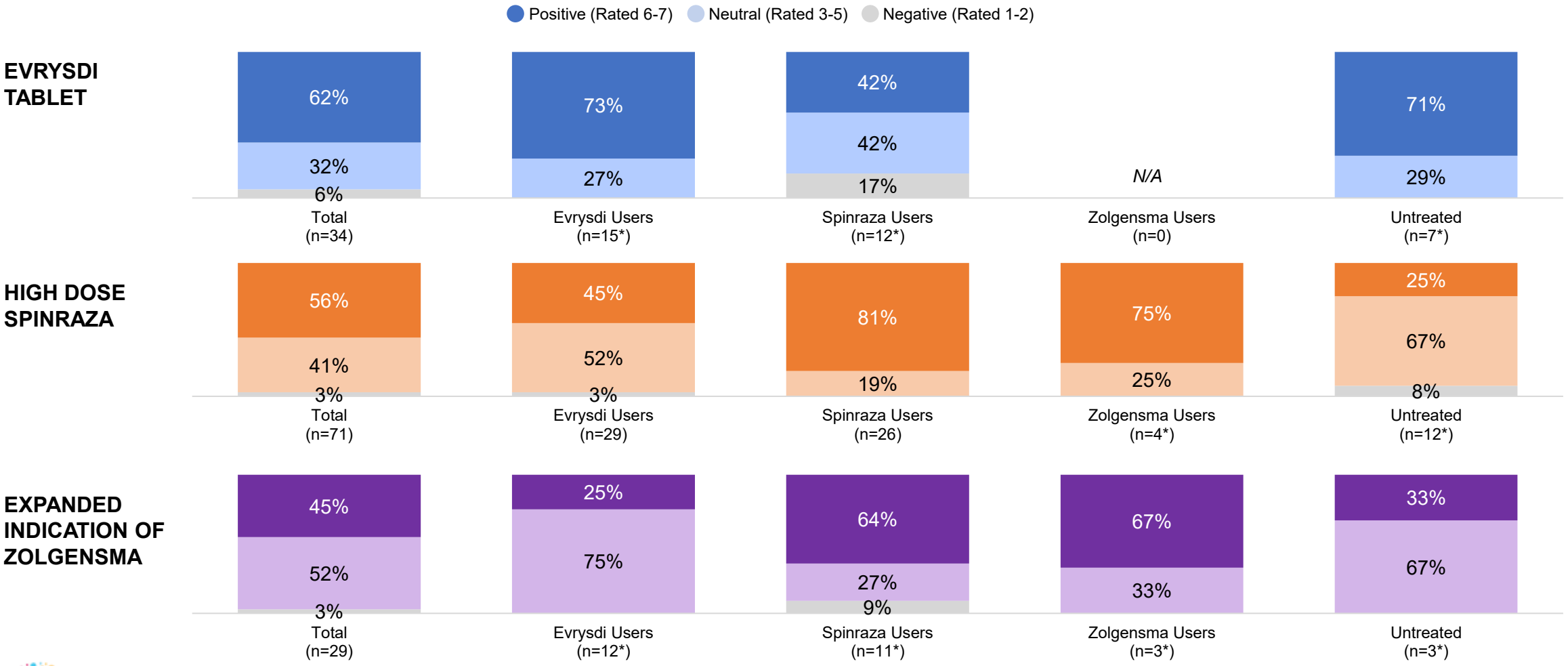
Base: Heard of New Formulation / Dosing Option (n=54) E3. Please list any new formulation or dosing options for existing SMA treatments you have heard of that are being developed.
Base: Total (n=100) E4. Which of the following new formulation or dosing options are being developed for Spinal Muscular Atrophy, if any, have you ever heard of?
Base: Aware of Treatment (Evrysdi Tablet; n=34, Spinraza High Dose; n=71, Zolgensma for 2-17; n=29) E5. What have you heard about Evrysdi Tablet / High Dose Spinraza / Expanded Indication of Zolgensma?

IMPRESSIONS OF TREATMENTS IN DEVELOPMENT

Mixed reactions to the new SMA treatments in development, however better impression of Tablet among the current Evrysdi Users and Untreated patients, whereas current Spinraza Users and Zolgensma had better impression of DEVOTE and STEER.

IMPRESSIONS OF TREATMENTS IN DEVELOPMENT

Among Those Aware of Treatment (Evrysdi Tablet; n=34, High Dose Spinraza; n=71, Expanded Zolgensma; n=29)
7-point scale

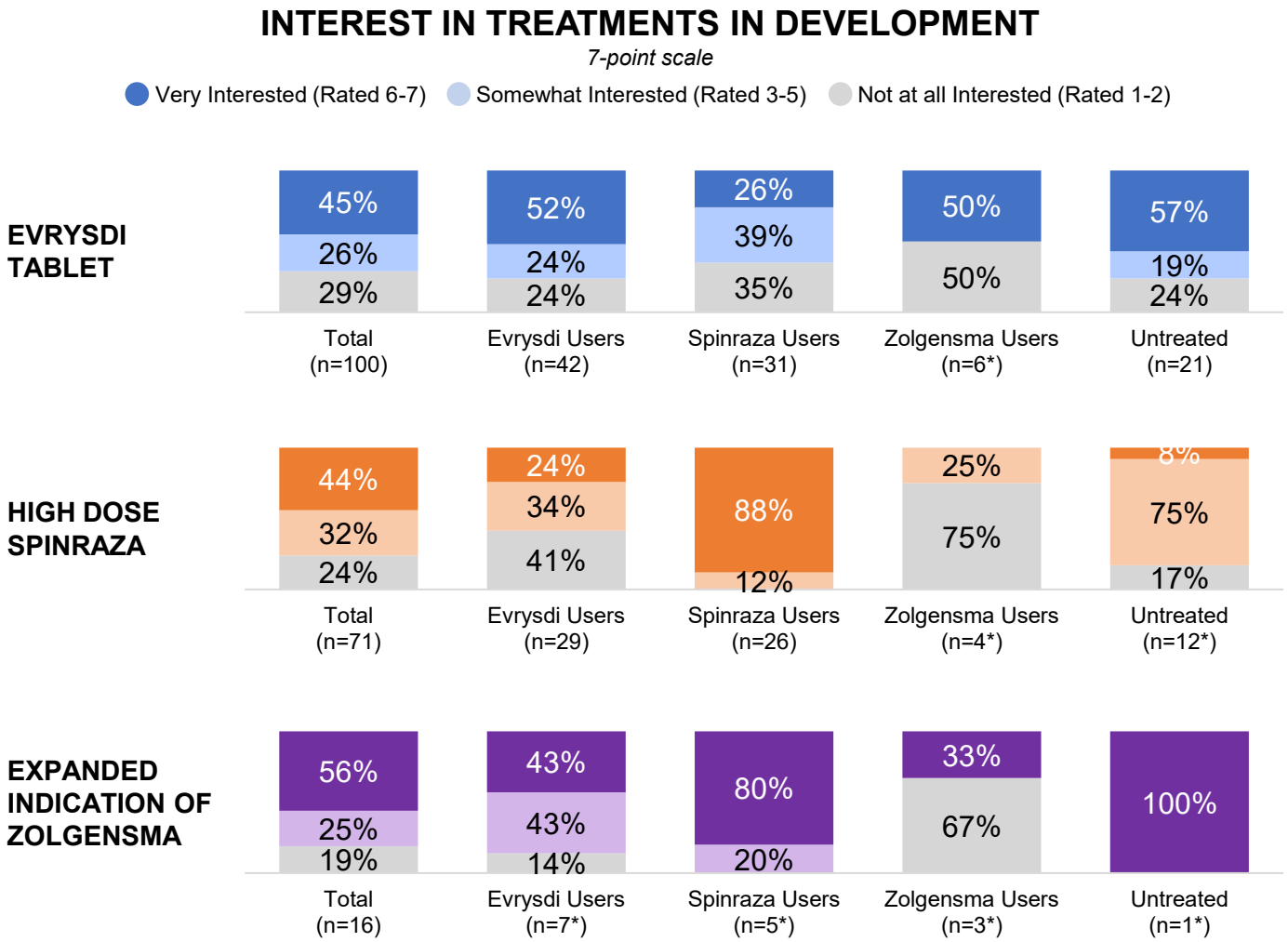


*CAUTION: SMALL BASE

Base: Aware of Treatment (Evrysdi Tablet; n=34, Spinraza High Dose; n=71, Zolgensma for 2-17; n=29) E6. Based on what you have heard, using a 7-point scale, where 1=Extremely Negative and 7=Extremely Positive, what is your overall impression of the following new formulation or dosing options that are being developed for Spinal Muscular Atrophy?

INTEREST IN TREATMENTS IN DEVELOPMENT

Multiple patient types have interest in Tablet and the expanded indication of Zolgensma, whereas only current Spinraza Users has higher interested in High Dose Spinraza .



DISCUSSION

Among Very Interested in Treatment in Development (Rated 5-7)
(Evrysdi Tablet; n=45, Spinraza High Dose; n=31, Zolgensma for 2-17; n=9)*

*"No need for refrigeration means I **can take a dose without any assistance**."*
– Adult Patient, Current Evrysdi User

*"I'm hoping it will **keep the drug effective** for the time between injections."*
– Adult Patient, Current Spinraza User

*"I think this is **groundbreaking** for all SMA patients in the community, especially if they are diagnosed later in toddlerhood."*
– Pediatric Caregiver, Current Zolgensma User

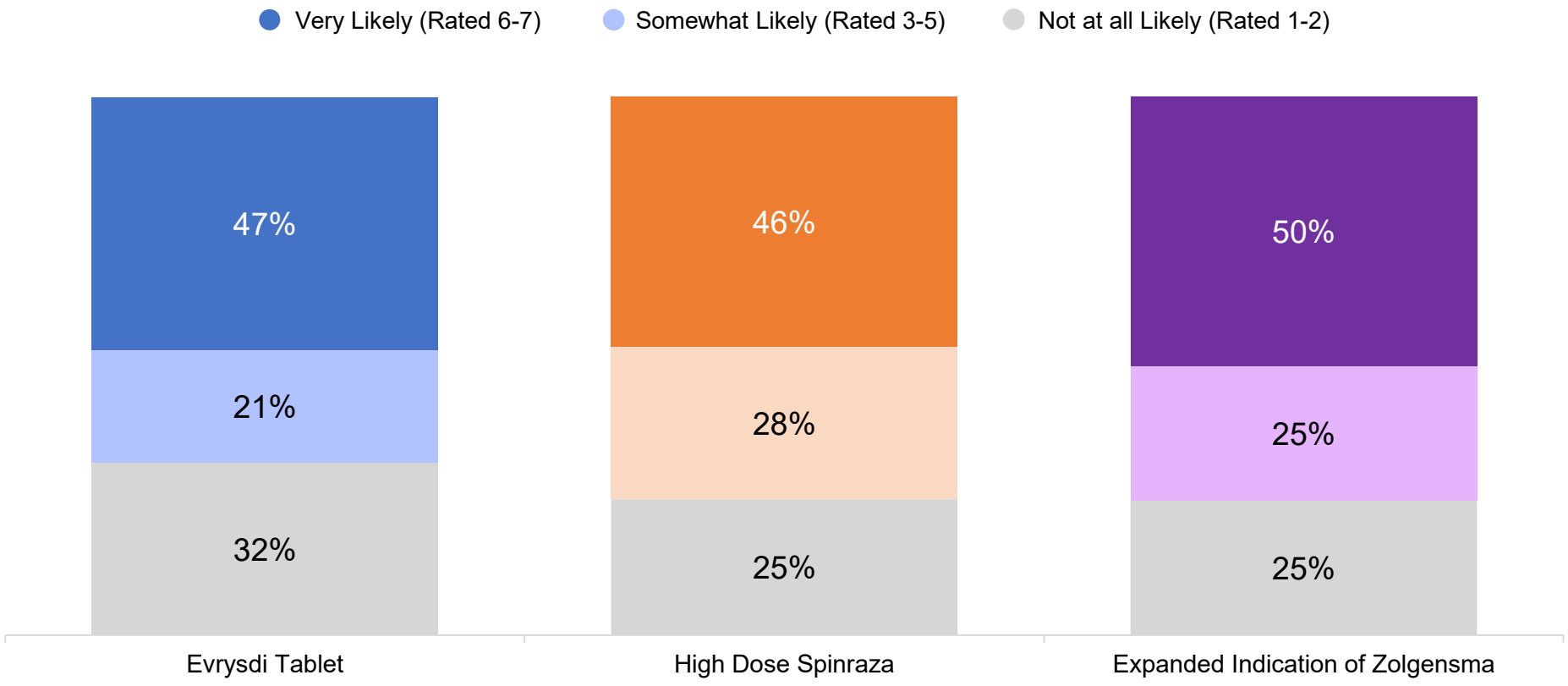
CAUTION: SMALL BASE Base: Total Patients (n=100) F2. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, what is your level of interest in the Evrysdi Tablet/High Dose Spinraza/Zolgensma expanding their indications for SMA patients ages 2-17 years old? / Base: Patients aware of High Dose Spinraza (n=71) F7. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, what is your level of interest in the High Dose Spinraza? / Base: Patients aware of and qualified for Expanded Indication of Zolgensma (n=16) F9. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, what is your level of interest in Zolgensma expanding their indications for SMA patients ages 2-17 years old? / Base: Very Interested in Tablet/High Dose Spinraza/Zolgensma for Ages 2-17 (Rated 5-7) (Evrysdi Tablet; n=45, High Dose Spinraza n=31, Zolgensma for Ages 2-17 years old n=9) F3b./F8b/F10b Why are you interested in the Evrysdi Tablet/High Dose Spinraza/Zolgensma indicated for SMA patients 2-17 years old?

FUTURE INTENTIONS WITH TREATMENTS IN DEVELOPMENT

Future intentions are mixed across all new SMA products in development.

LIKELIHOOD TO TALK TO DOCTOR ABOUT FUTURE TREATMENT

Among Aware of Future Treatment (Evrysdi Tablet; n=34, Spinraza High Dose; n=71, Zolgensma for ages 2-17; n=16)
7-Point Scale



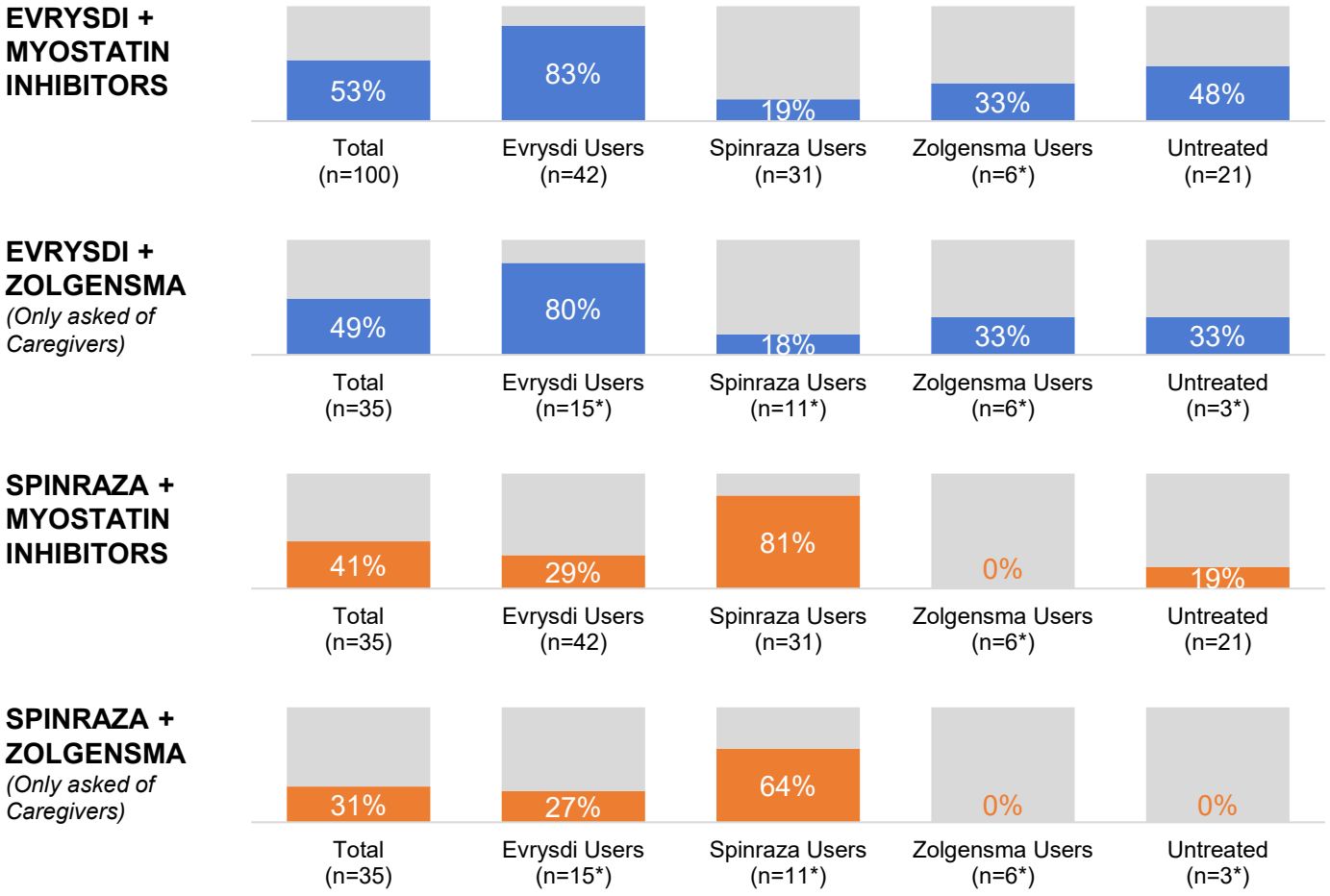
Base: Aware of Future Treatment (Evrysdi Tablet; n=34, Spinraza High Dose; n=71, Zolgensma for ages 2-17; n=16) E7. If the following new formulation or dosing options became available, how likely would you be to ask your doctor to switch to/try the following medicine?

COMBINATION THERAPY INTEREST

Evrysdi Users have higher interest in combinations with Evrysdi, and Spinraza Users have higher interest in combinations with Spinraza. Untreated were more interested in combinations that included Evrysdi plus Myostatin Inhibitors or Zolgensma.

INTEREST IN COMBINATION THERAPY

7-point scale, rated 6-7



DISCUSSION

Reasons for Interest/No Interest in Combination Therapy

*"They address **two different targets of treatment,** so it seems likely that they would be more effective taken together."*
– Adult Patient, Current Spinraza User

*"Adding a new treatment to Evrysdi seems like a good way to **improve versus maintain.**"*
– Pediatric Caregiver, Current Evrysdi User

*"They address two different types of treatment by **addressing the SMN protein and also blocking a muscle inhibitor**. Since Spinraza has been ineffective for me, I feel the two in combination would be even more effective."*
– Adult Patient, Current Spinraza User

*"If it has a positive impact and the data show it's **beneficial and safe**, then I'd be interested."*
– Pediatric Caregiver, Current Spinraza User

*CAUTION: SMALL BASE

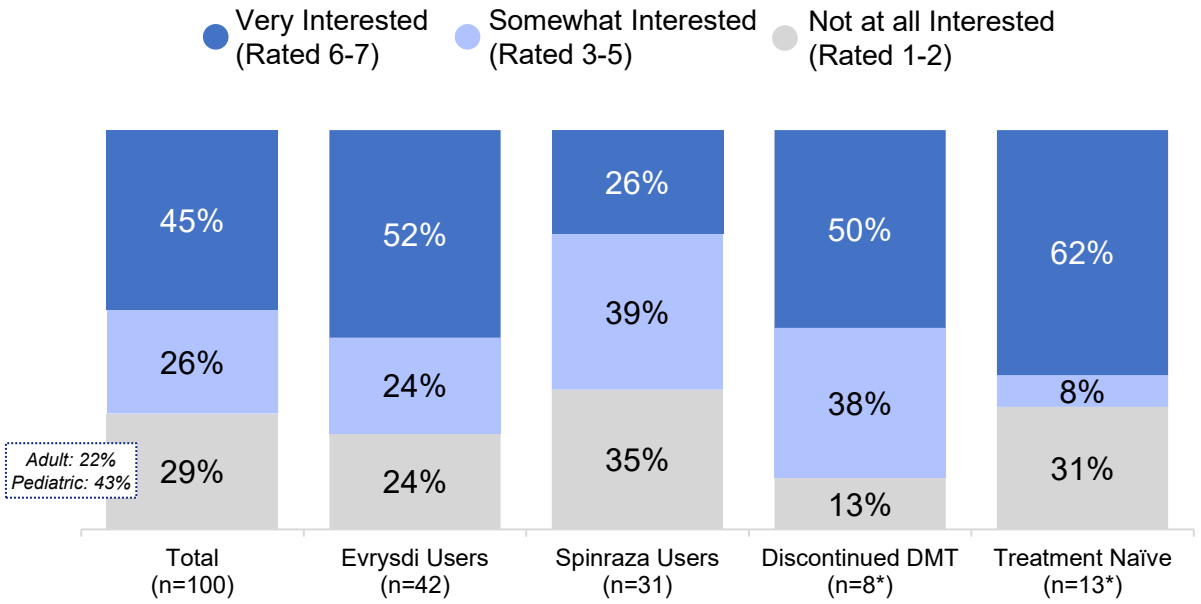
Base: Total Patients (n=100) H1. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, how interested are you in the following SMA treatment combinations in the future? / Base: Not Interested in Combination Therapy / H2A. Why are you NOT interested in [COMBINATION]? / H2B. Why are you interested in [COMBINATION]?

TPP REVIEW

Majority of patients are interested in the Evrysdi Tablet across patient types, primarily due to the convenience and simple administration.

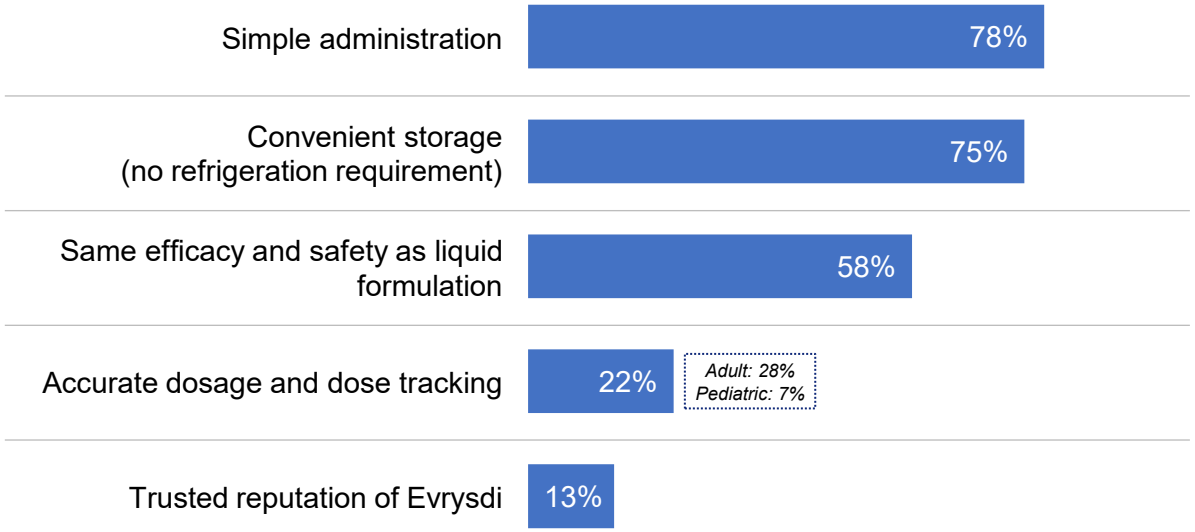
INTEREST IN EVRYSDI TABLET BY USER GROUP

Among Total Patients (n=100)
7-Point Scale



REASONS TO SWITCH TO TABLET

Among Very Interested in Tablet (Rated 5-7) (n=55)



*"It's **easier to take a** pill than it is to get bottle out of fridge and then draw up the right amount in a syringe."* – Adult Patient, Current Evrysdi User

*"**The dosage would be correct** rather than approximate, I would **not have to store in the refrigerator**, and there is an **option to have it dissolved in water.**"* – Adult Patient, Current Spinraza User

*"I have no problem swallowing and I **won't have to worry** about refrigeration or bringing a cooler when traveling."* – Adult Patient, Current Evrysdi User

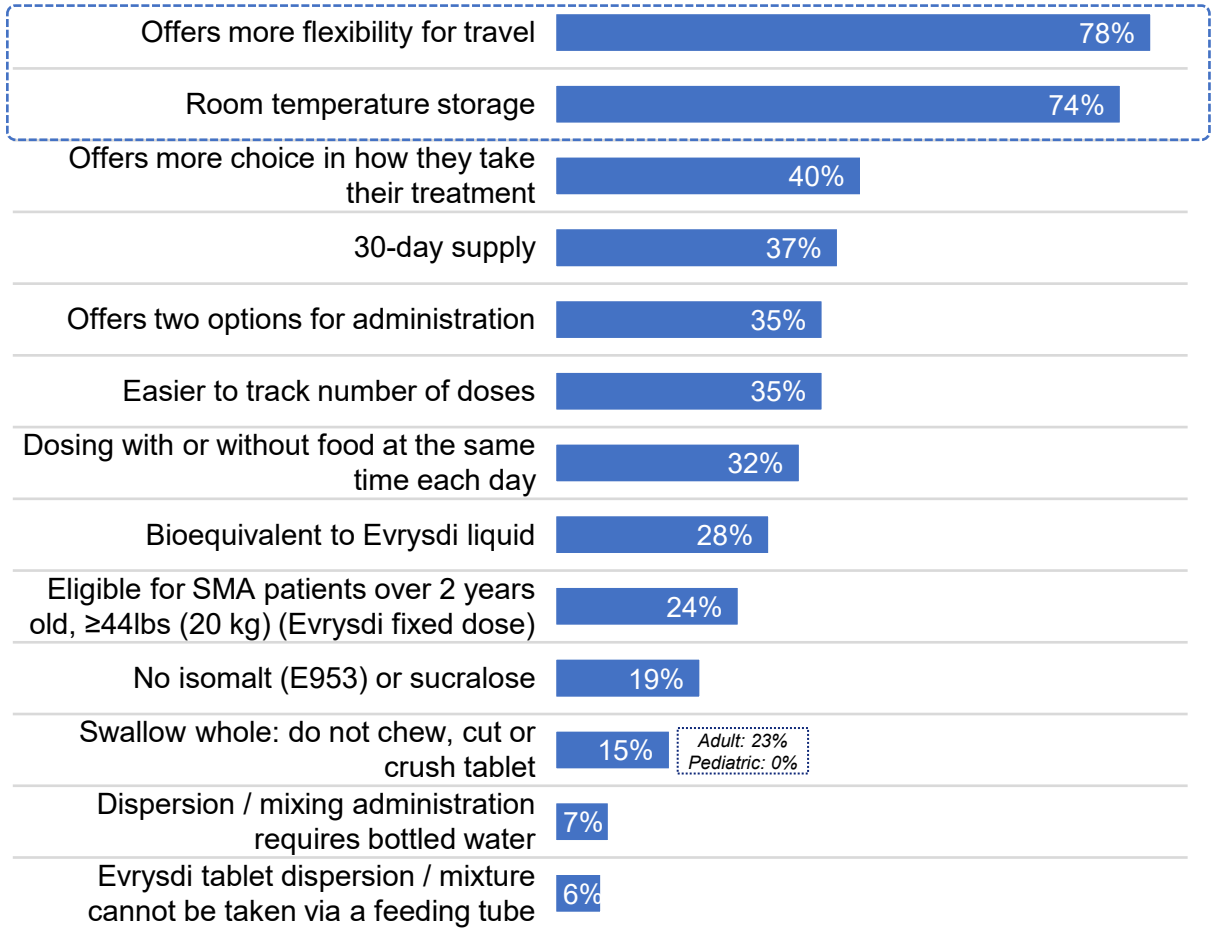
*CAUTION: SMALL BASE
Base: Total Patients (Evrysdi Users; n=42, Spinraza Users; n=31, Discontinued DMT; n=8*, Treatment Naïve n=13*) F2. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, what is your level of interest in the Evrysdi Tablet? / Base: Extremely Interested in Tablet (Rated 5-7) (n= 45) F3a. Why are you interested in the Evrysdi Tablet? / F3C. Which of the following reasons would you switch to the Evrysdi tablet? / Base: Not Interested in Evrysdi Tablet (Rated 1-3) (n=38) F3b. Why are you NOT interested in the Evrysdi Tablet?

TPP REVIEW

Top perceived advantages are convenience and flexibility – more options to fit their daily living. The main perceived disadvantages are focused on the administration difficulties, especially among caregivers of pediatric patients.

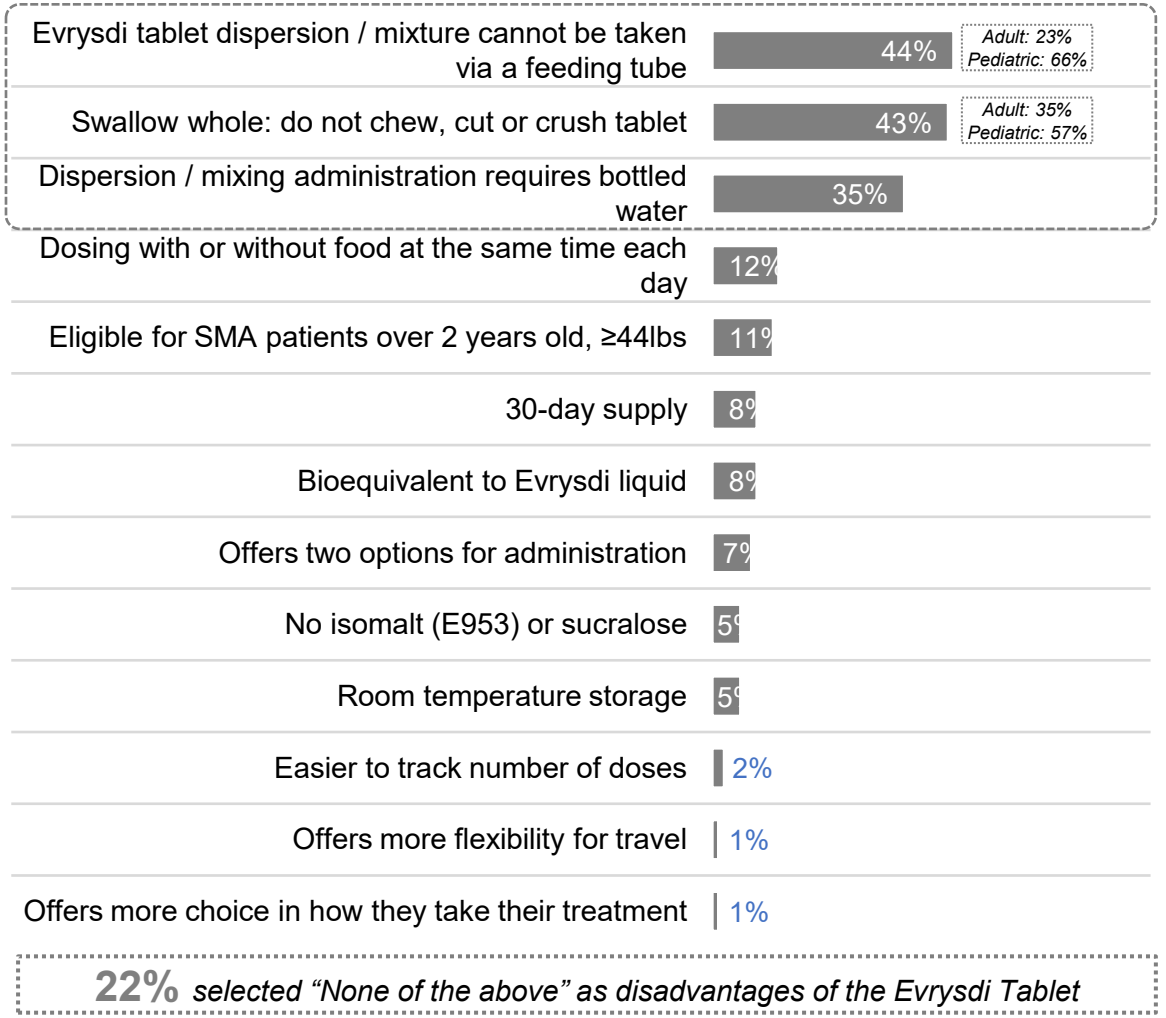
TOP EVRYSDI TABLET ADVANTAGES

Among Total Patients (n=100)



TOP EVRYSDI TABLET DISADVANTAGES

Among Total Patients (n=100)



22% *selected "None of the above" as disadvantages of the Evrysdi Tablet*

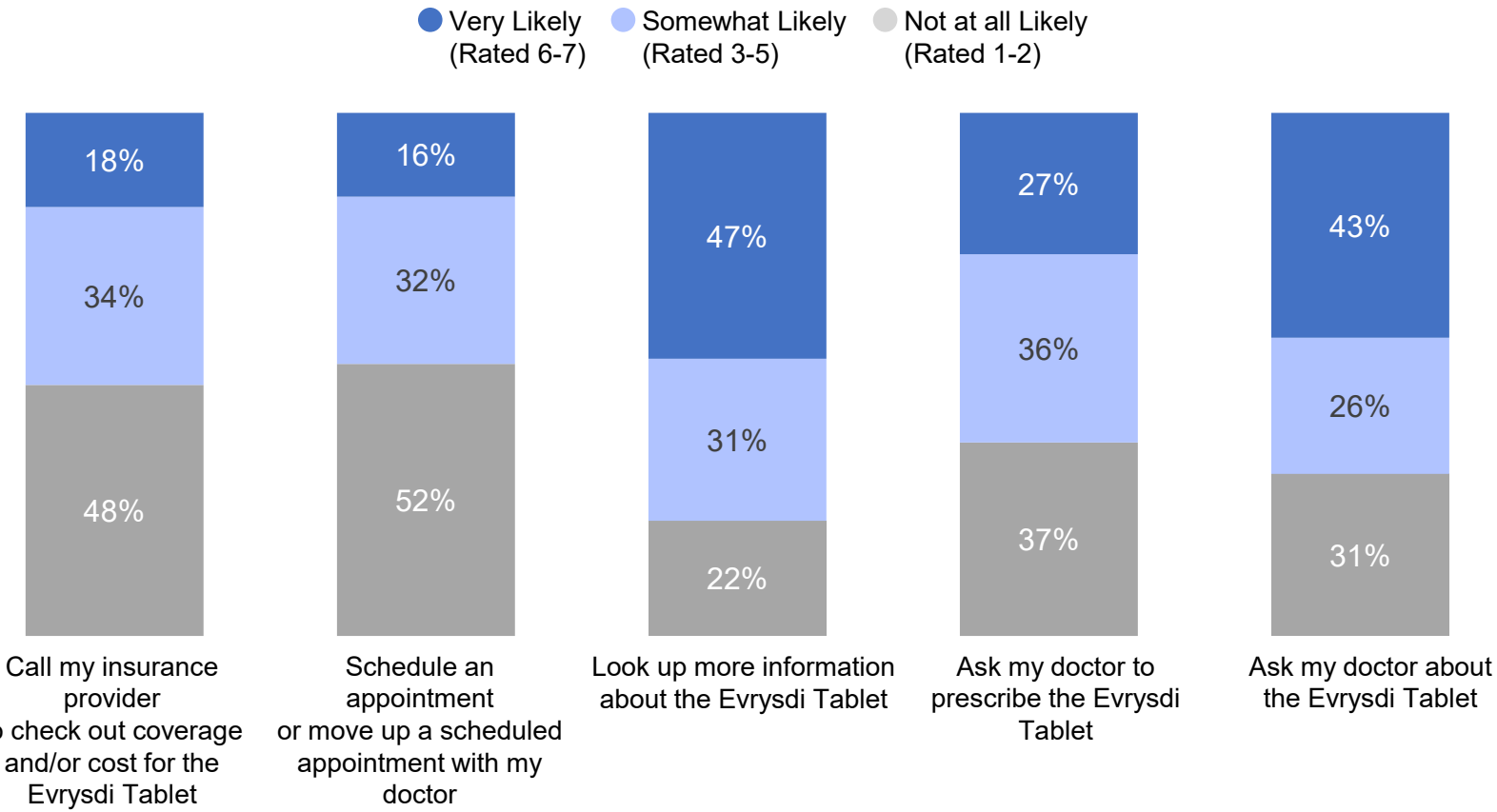
Base: Total Patients (n=100) F3. What are the primary advantages and disadvantages of the Evrysdi Tablet for you? Please select up to 5 features in each column.

TPP REVIEW

Patients are more likely to seek more information about Evrysdi Tablet before being more proactive in requesting it from their doctor, office, and insurance.

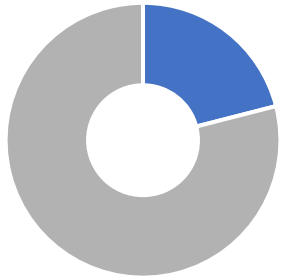
EVRYSDI TABLET: LIKELIHOOD TO ACT

Among Total (n=100)



IMPACT OF TABLET FEATURES ON OVERALL IMPRESSION

Among Total (n=100)
7-point scale



21% *Adult: 14% Pediatric: 34%*

Reported Extreme Impact on Overall Impression of Evrysdi Tablet **If Tablet Dispersion Cannot Be Taken Through N/G Tube**

Current Evrysdi Users (n=42): 36%
Current Spinraza Users (n=31): 13%
Treatment Naïve (n=13): 0%

24%

Reported Extreme Impact on Overall Impression of Evrysdi Tablet **If Tablet Was Only Approved to be Swallowed Whole**

Current Evrysdi Users (n=42): 38%
Current Spinraza Users (n=31): 10%
Treatment Naïve (n=13): 15%

Base: Total Patients (n=100) F4. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely would you do the following? / F5. If the Evrysdi Tablet could not be used with a nasogastric tube/gastrostomy tube, how much of an impact, if any, does that have on your overall impression of the product. Using a 7-point scale, where 1 means “No impact” and 7 means “Extreme Impact”. / F6. If the Evrysdi Tablet was only approved to be swallowed whole with water, what impact would that have on your overall impression of the product, if any? Using a 7-point scale, where 1 means “No impact” and 7 means “Extreme Impact”.

APPENDIX

PATIENTS

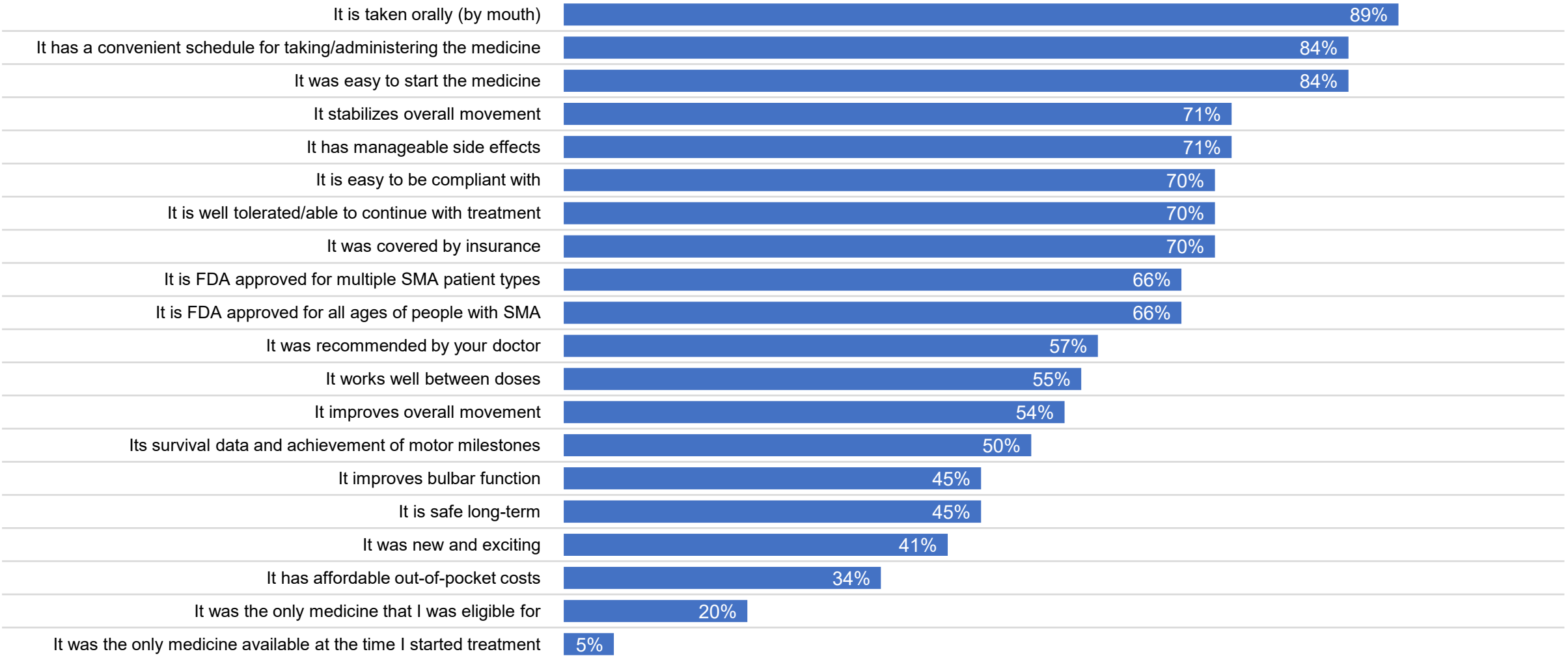
Evrysdi® risdiplam 60 mg powder for oral solution

USAGE DRIVERS: EVRYSDI

Convenience are top reasons to use Evrysdi.

TOP REASONS TO USE EVRYSDI

Current or Past Evrysdi Users (n=56)
% Selected



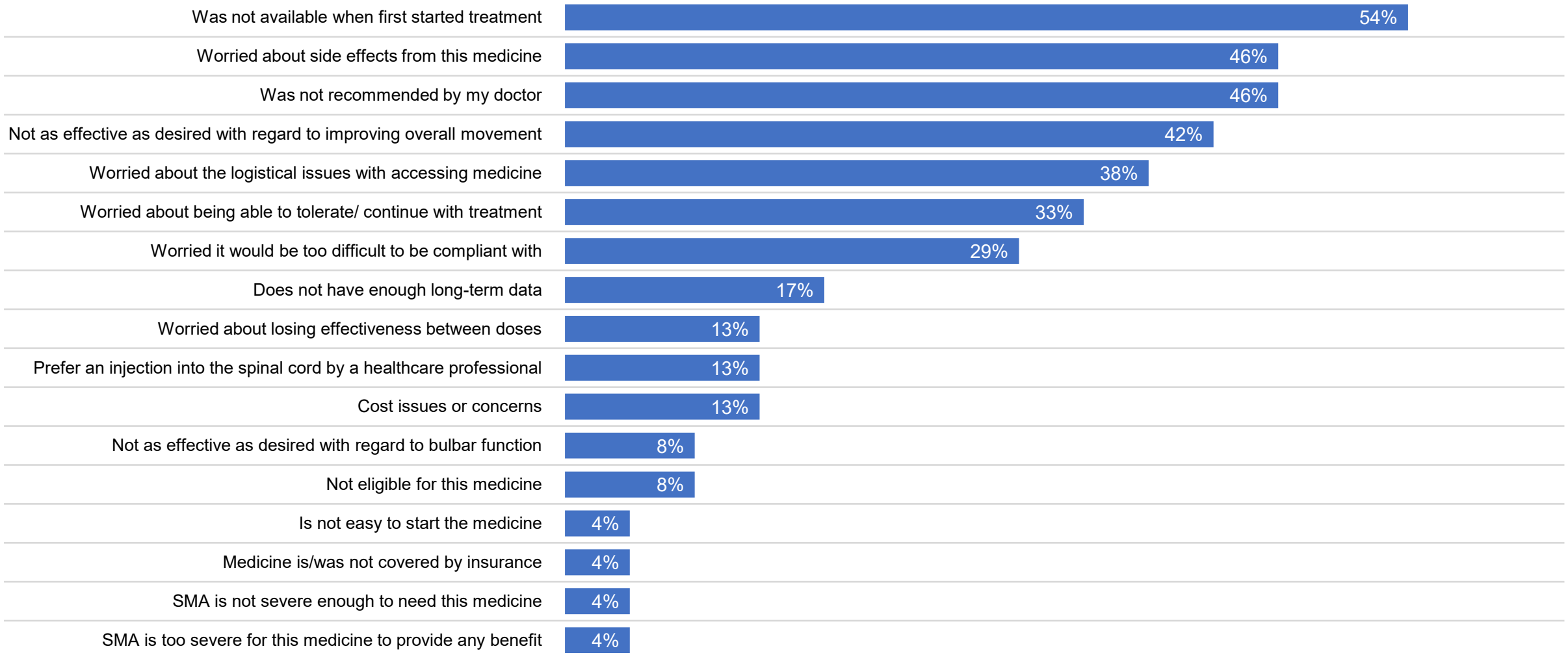
Base: Currently or Previously Taken Evrysdi (n=56)
B1. For which of the following reasons did you choose to use the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

USAGE BARRIERS: EVRYSDI

Main reasons for not using Evrysdi was the lack of availability, side effects and not being recommended by doctor.

TOP REASONS FOR NEVER USING EVRYSDI

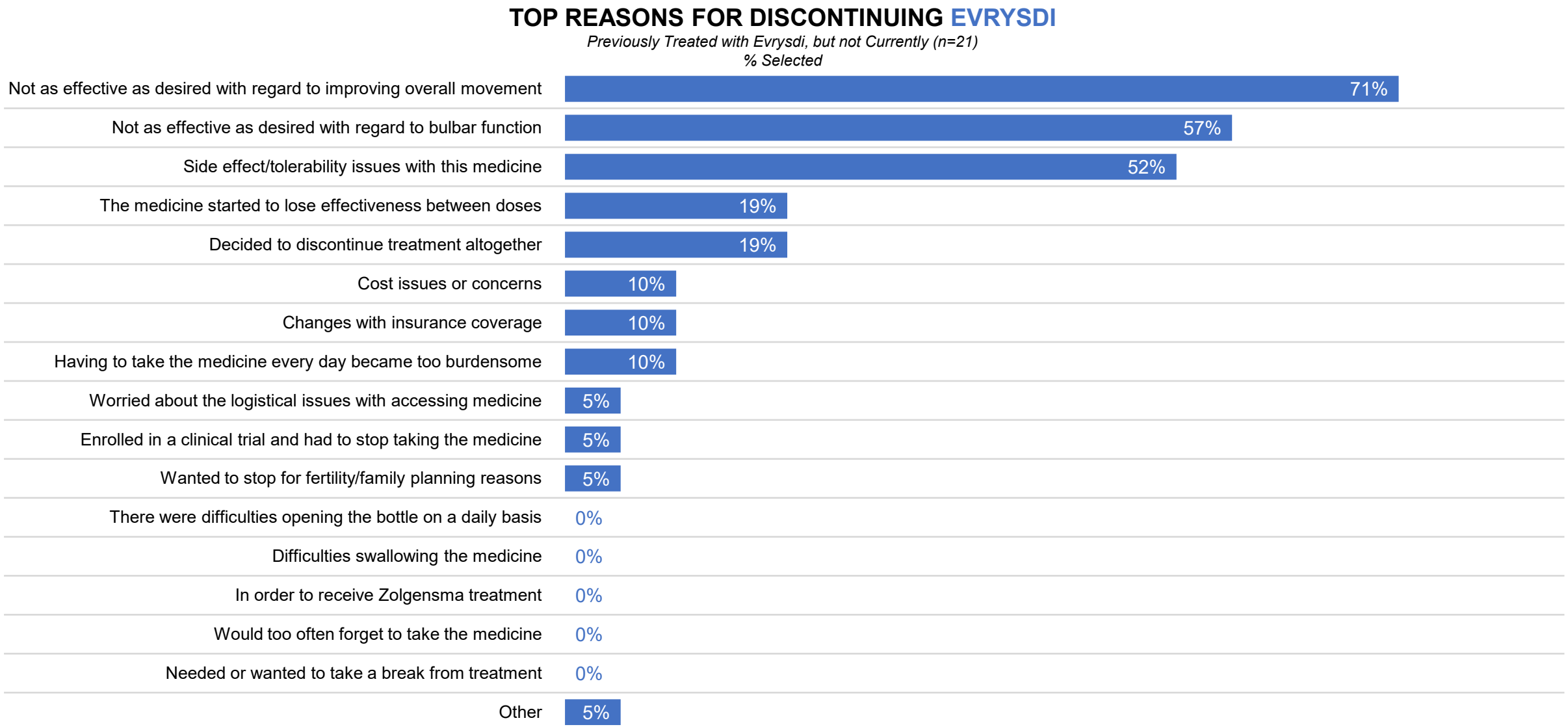
Currently or Previously Treated but Never Treated with Evrysdi (n=24)
% Selected



Base: Currently or Previously Treated but Never Treated with Evrysdi (n=24) or Spinraza (n=26)
B2. For which of the following reasons have you never used the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

DISCONTINUATIONS: EVRYSDI

Efficacy is the primary reason to discontinue Evrysdi.



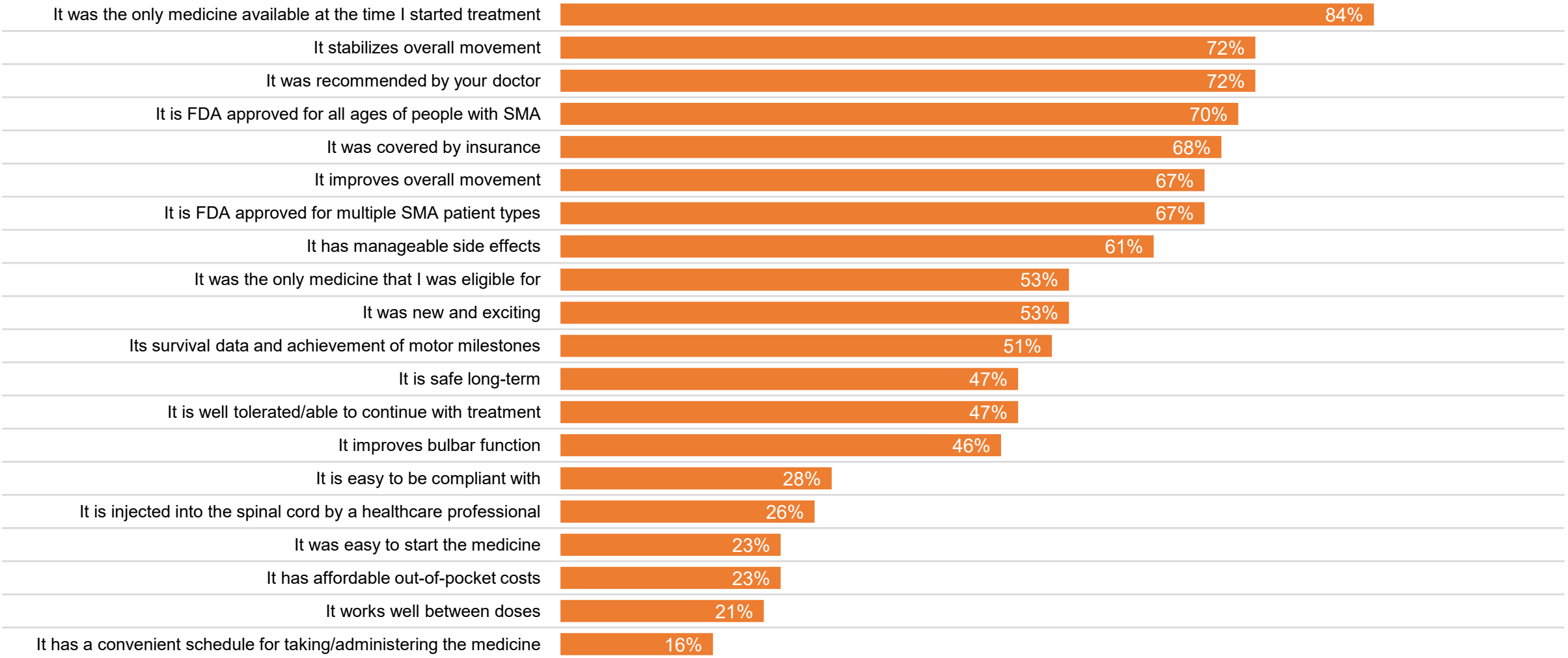
Base: Previously Treated with Evrysdi, but not Currently (n=21)
C2. For which of the following reasons did you stop using the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

USAGE DRIVERS: SPINRAZA

Top reasons to use Spinraza is that it was the only treatment available, in addition to overall movement stabilization, recommended by doctor, and approved for all ages.

TOP REASONS TO USE SPINRAZA

Current or Past Spinraza Users (n=57)
% Selected



Evrysdi risdiplam

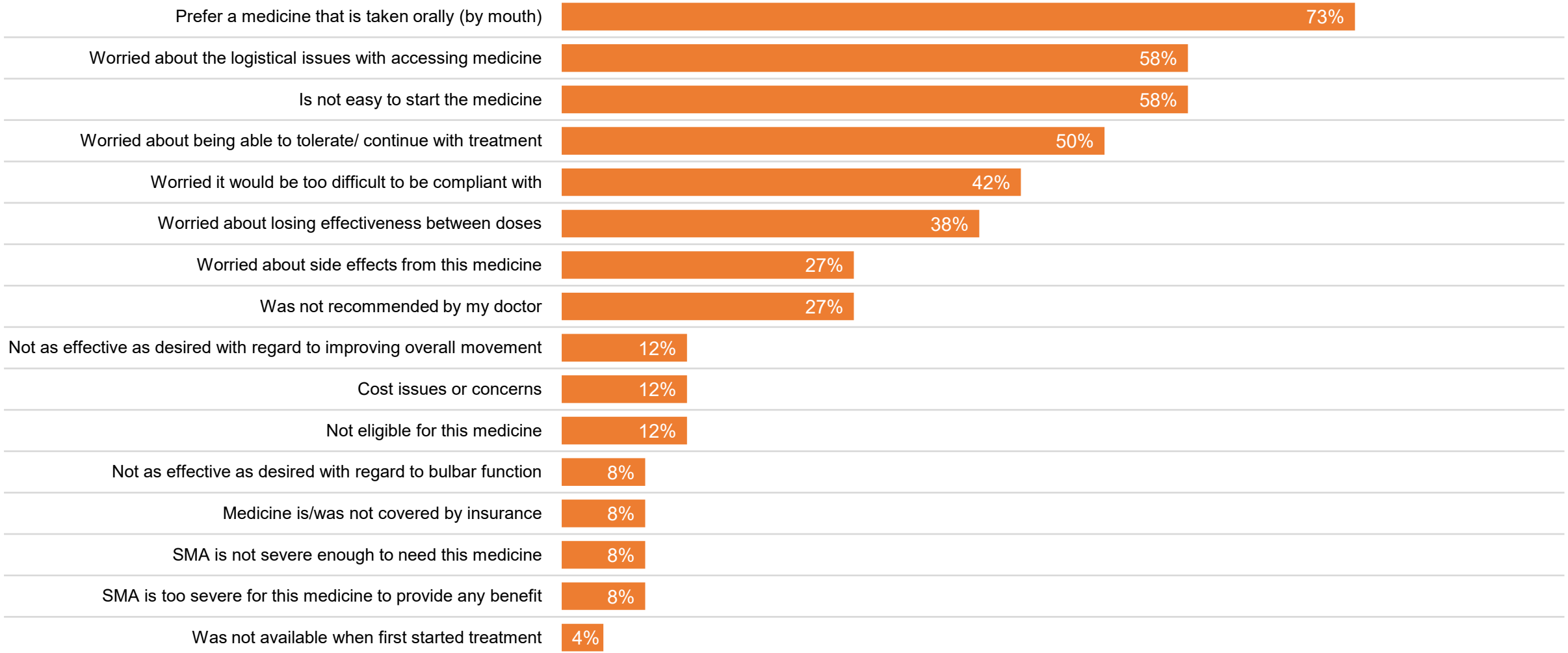
Base: Currently or Previously Taken Spinraza (n=57)
B1. For which of the following reasons did you choose to use the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

USAGE BARRIERS: SPINRAZA

Burdensome administration and logistics are top reasons for never using Spinraza.

TOP REASONS FOR NEVER USING SPINRAZA

Currently or Previously Treated but Never Treated with Spinraza (n=26)
% Selected



Evrysdi risdiplam

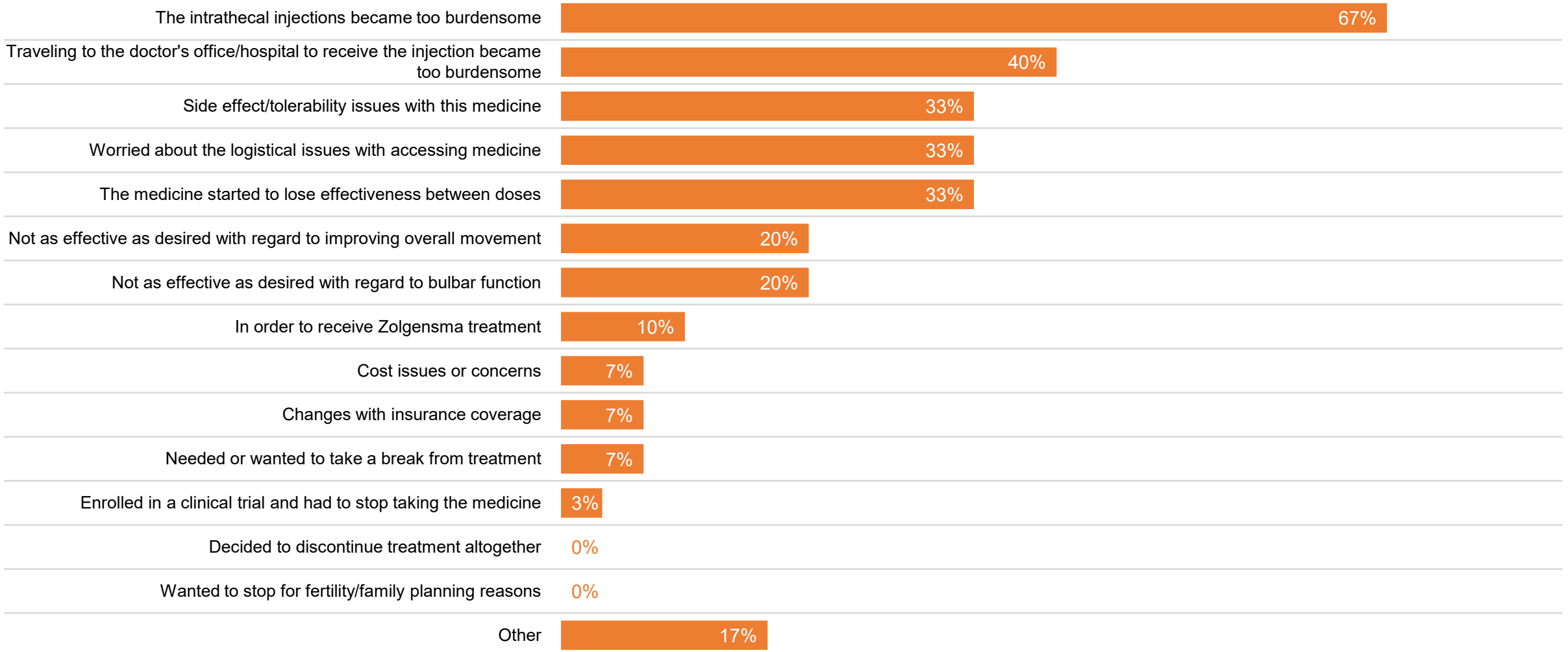
Base: Currently or Previously Treated but Never Treated with Evrysdi (n=24) or Spinraza (n=26)
B2. For which of the following reasons have you never used the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

DISCONTINUATIONS: SPINRAZA

The burden of intrathecal injections is the primary reason to discontinue Spinraza.

TOP REASONS FOR DISCONTINUING SPINRAZA

Previously Treated with Spinraza, but not Currently (n=30)
% Selected



Evrysdi risdiplam

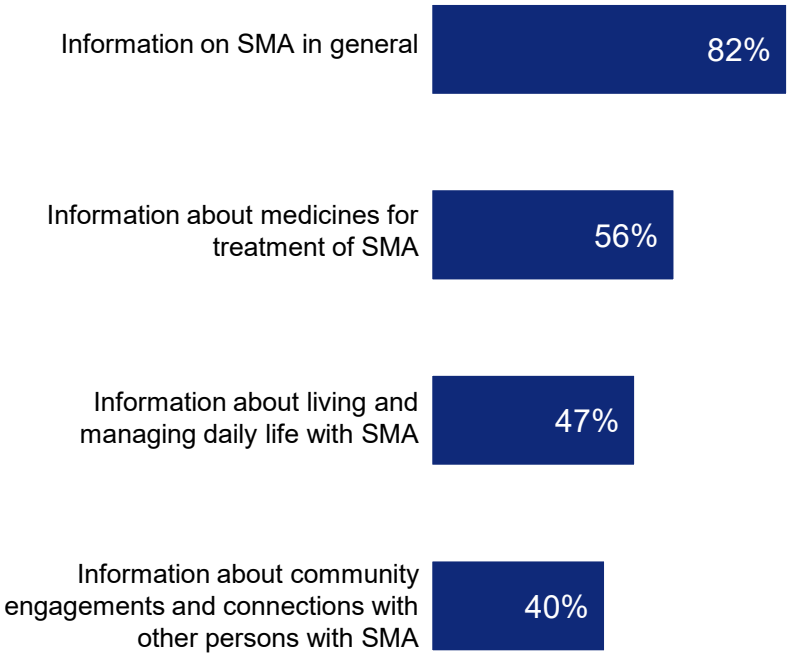
Base: Previously Treated with Spinraza, but not Currently (n=30)
C2. For which of the following reasons did you stop using the following medicine(s) for the treatment of spinal muscular atrophy (SMA)?

SOURCES OF INFORMATION

All types of information about SMA is of interest and patients utilize a variety of sources from digital (Facebook, online forums) and their doctor and advocacy groups.

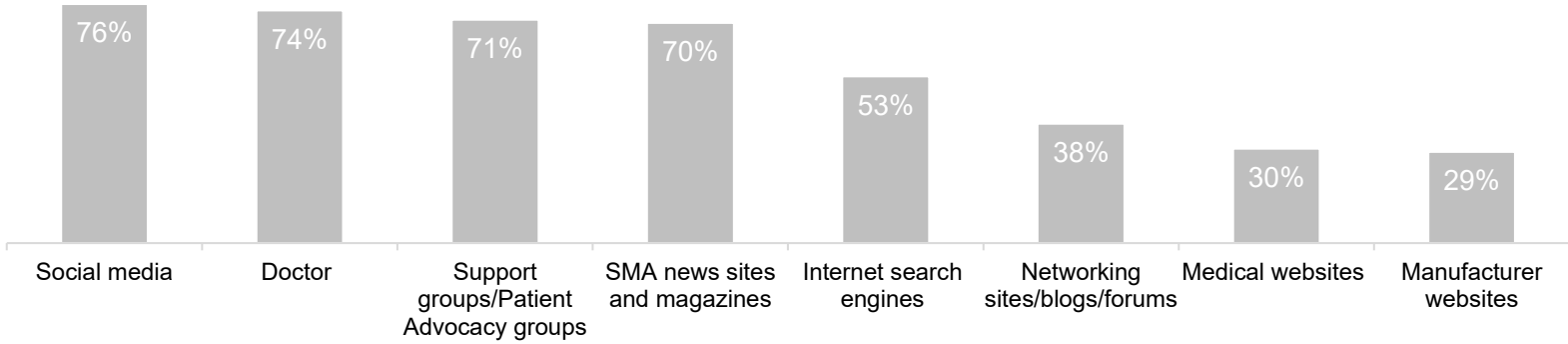
SMA INFORMATION OF INTEREST

Among Total (n=100)



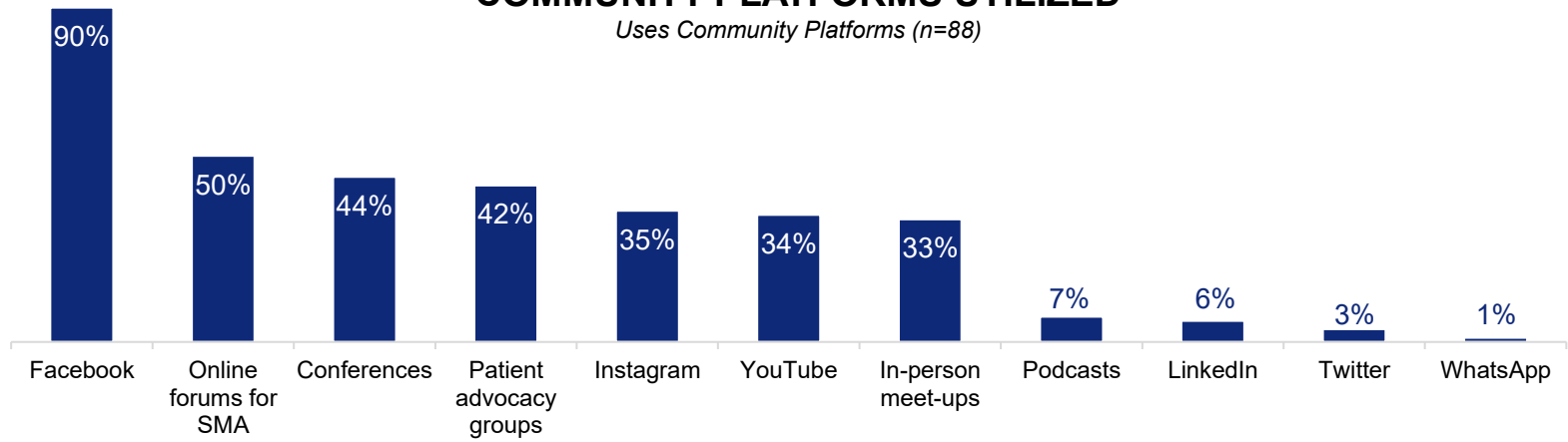
SOURCES OF INFORMATION

Among Total (n=100)



COMMUNITY PLATFORMS UTILIZED

Uses Community Platforms (n=88)



Base: Total Patients (n=100) D1. What information related to spinal muscular atrophy (SMA) are you / would you be interested in learning about? / D2. What sources of information do you use to learn about spinal muscular atrophy? / Base; Uses Community Platforms (n=88) D3. Which of the following community platforms do you use to learn and communicate about SMA?

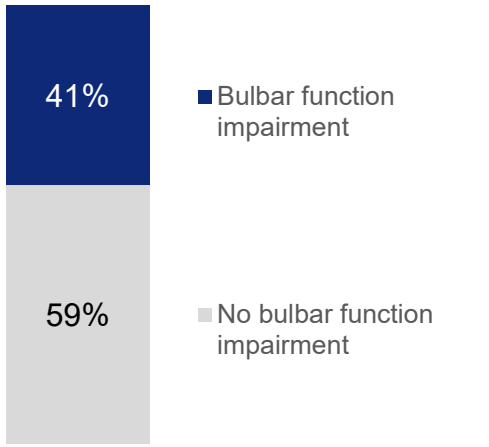
APPENDIX

HCPs

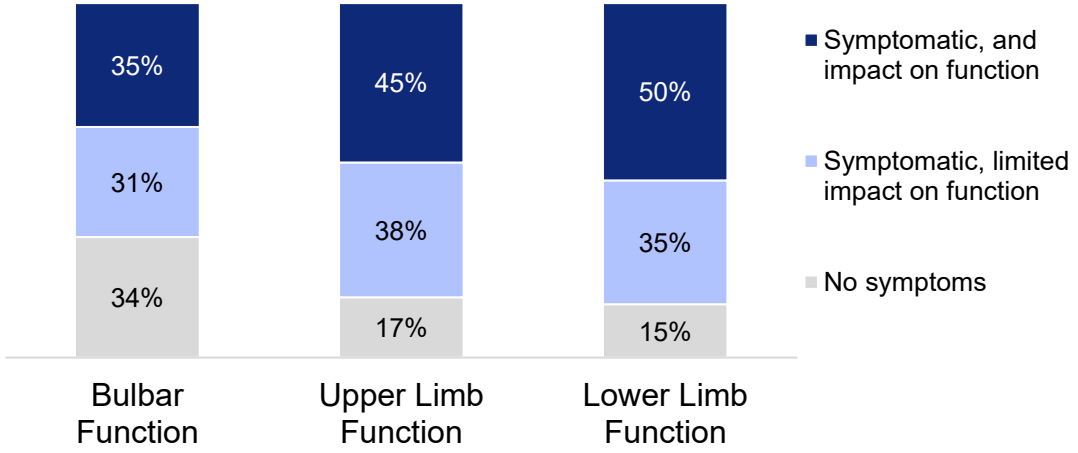
PATIENT POPULATION SNAPSHOT

SMA symptoms vary across the overall patient population as reported by HCPs, aligning with the even distribution of patient types 1 through 4. There is an even split of patients using Commercial insurance and federal health insurance programs (Medicare and Medicaid.)

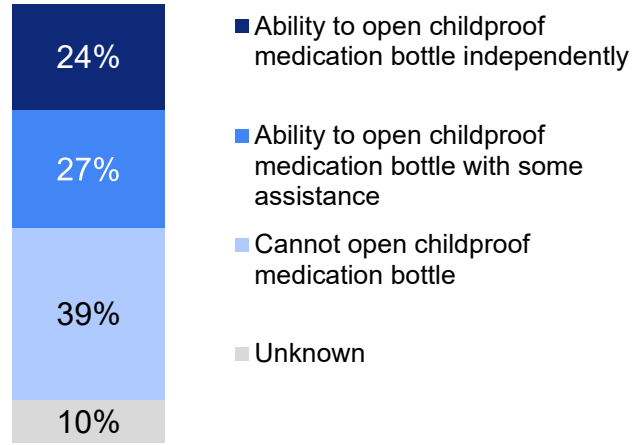
BULBAR IMPAIRMENT



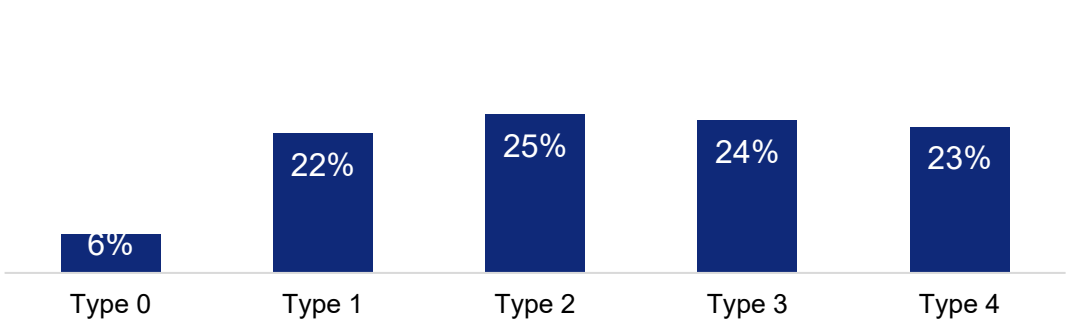
SMA SYMPTOMS



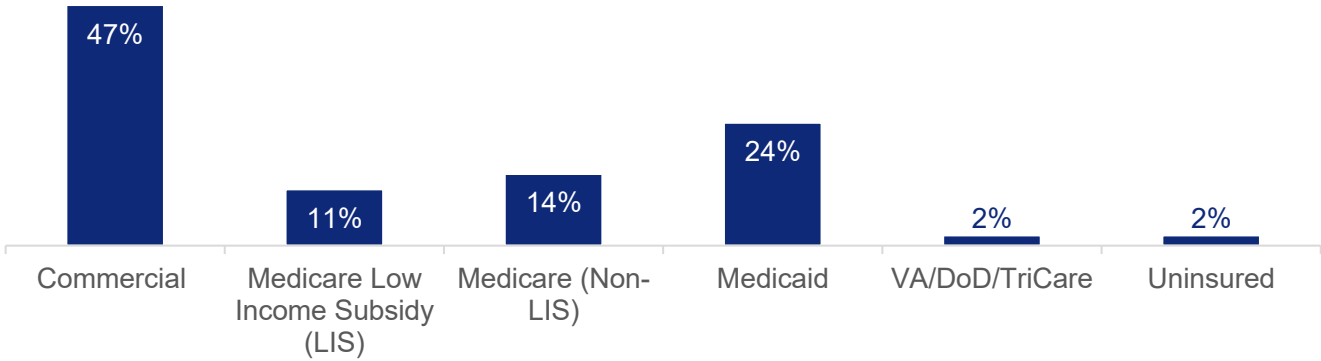
ABILITY TO OPEN PILL BOTTLE



SMA TYPE



INSURANCE



Base: Total HCPs (n=100); Pediatric Neurologists (n=43), Adult Neurologists (n=57) A2. What percent of your spinal muscular atrophy (SMA) patients fall into the following bulbar function categories? / A3. What percent of your spinal muscular atrophy (SMA) patients fall into the following SMA Type categories? / A4. What percent of your spinal muscular atrophy (SMA) patients fall into the following insurance categories? / A5. What percent of your spinal muscular atrophy (SMA) patients fall into the following categories? / A6. What percent of your spinal muscular atrophy (SMA) patients fall into the following categories?

APPENDIX

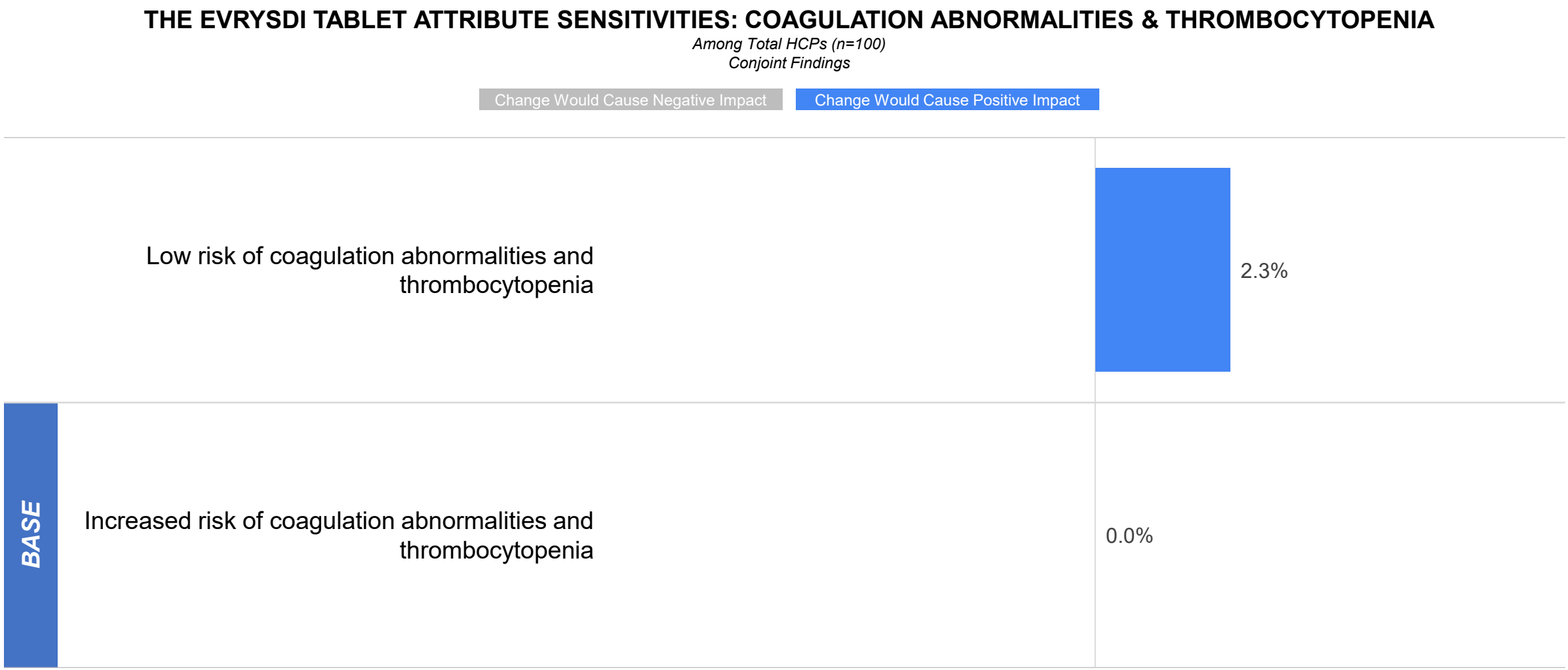
HCPs: ATTRIBUTE SENSITIVITY & UTILITY

CONJOINT FINDINGS

TABLET ATTRIBUTE SENSITIVITIES: COAGULATION THROMBOCYTOPENIA

HCP

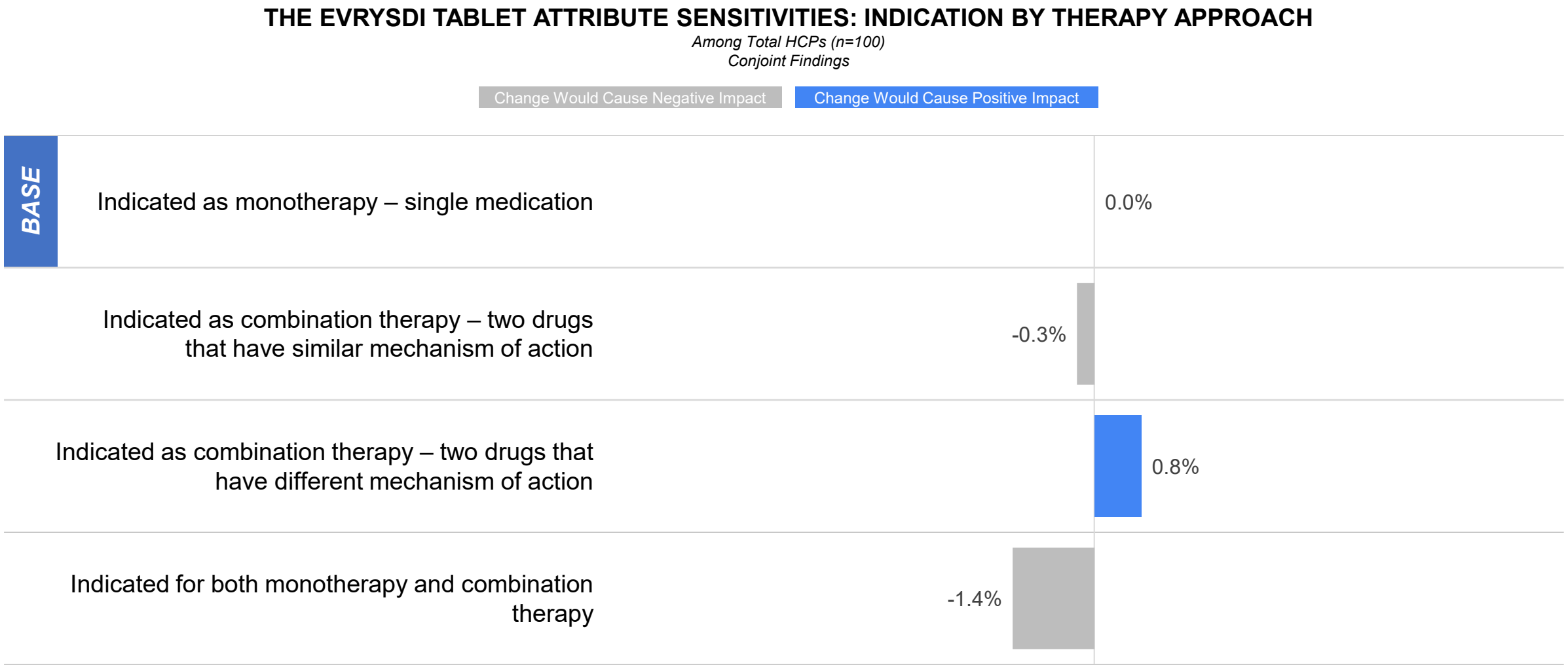
Coagulation abnormalities and thrombocytopenia is an acceptable risk for SMA.



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: INDICATION BY THERAPY APPROACH

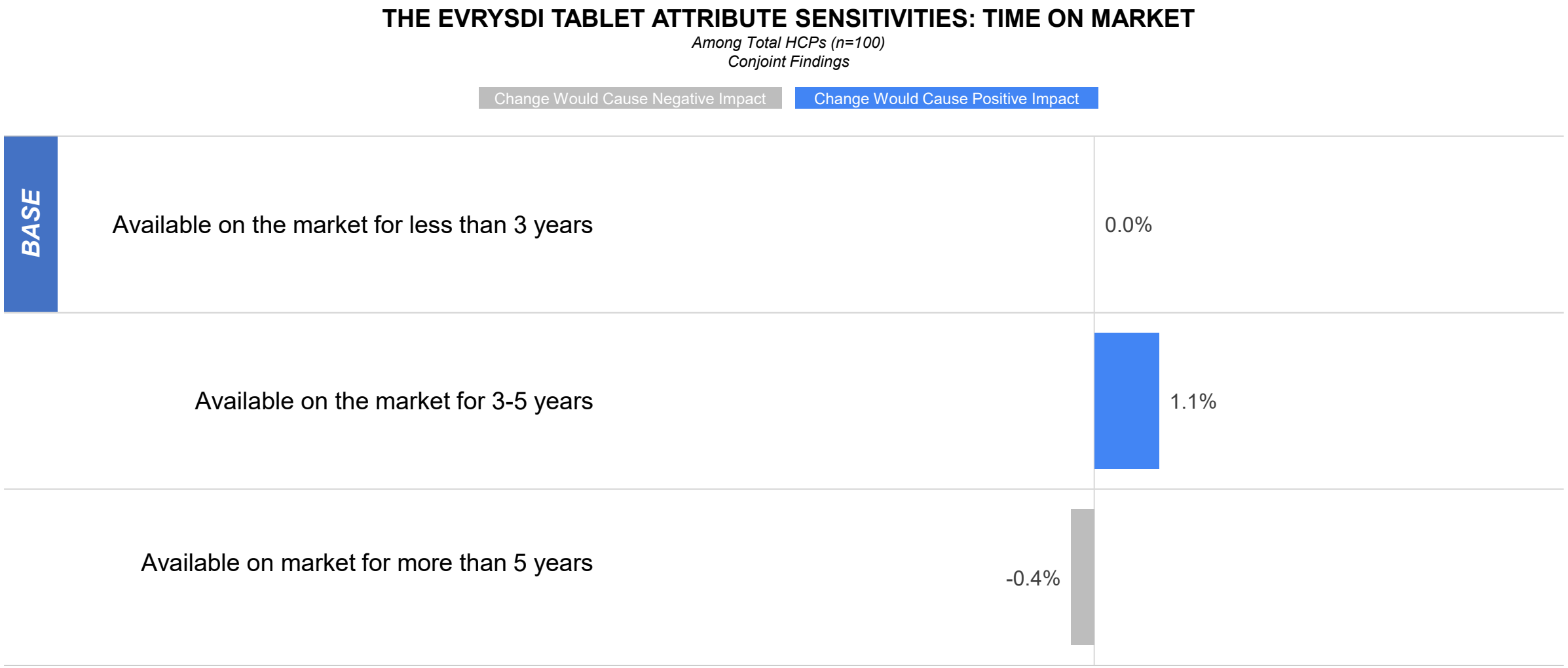
Combination with different mechanism of action is more preferred than similar mechanism of action.



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: TIME ON MARKET

Time on market creates a polarizing share – if too short or too long on market, both have a negative impact.



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: EFFECTS ON MALE FERTILITY

Effects on male fertility has minimal impact on share.

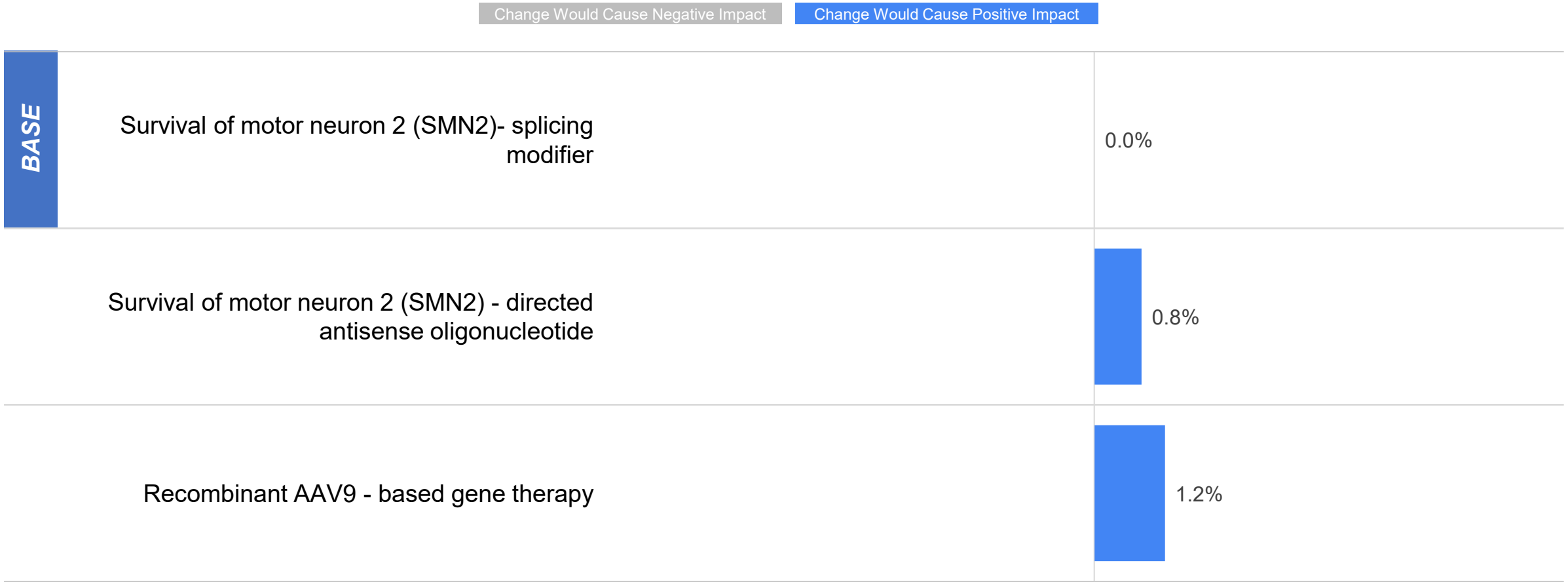
Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: MECHANISM OF ACTION

SMN2 mechanism of action has minimal impact on share. Evrysdi mechanism of action has lower familiarity than Spinraza mechanism of action.

THE EVRYSDI TABLET ATTRIBUTE SENSITIVITIES: MECHANISM OF ACTION

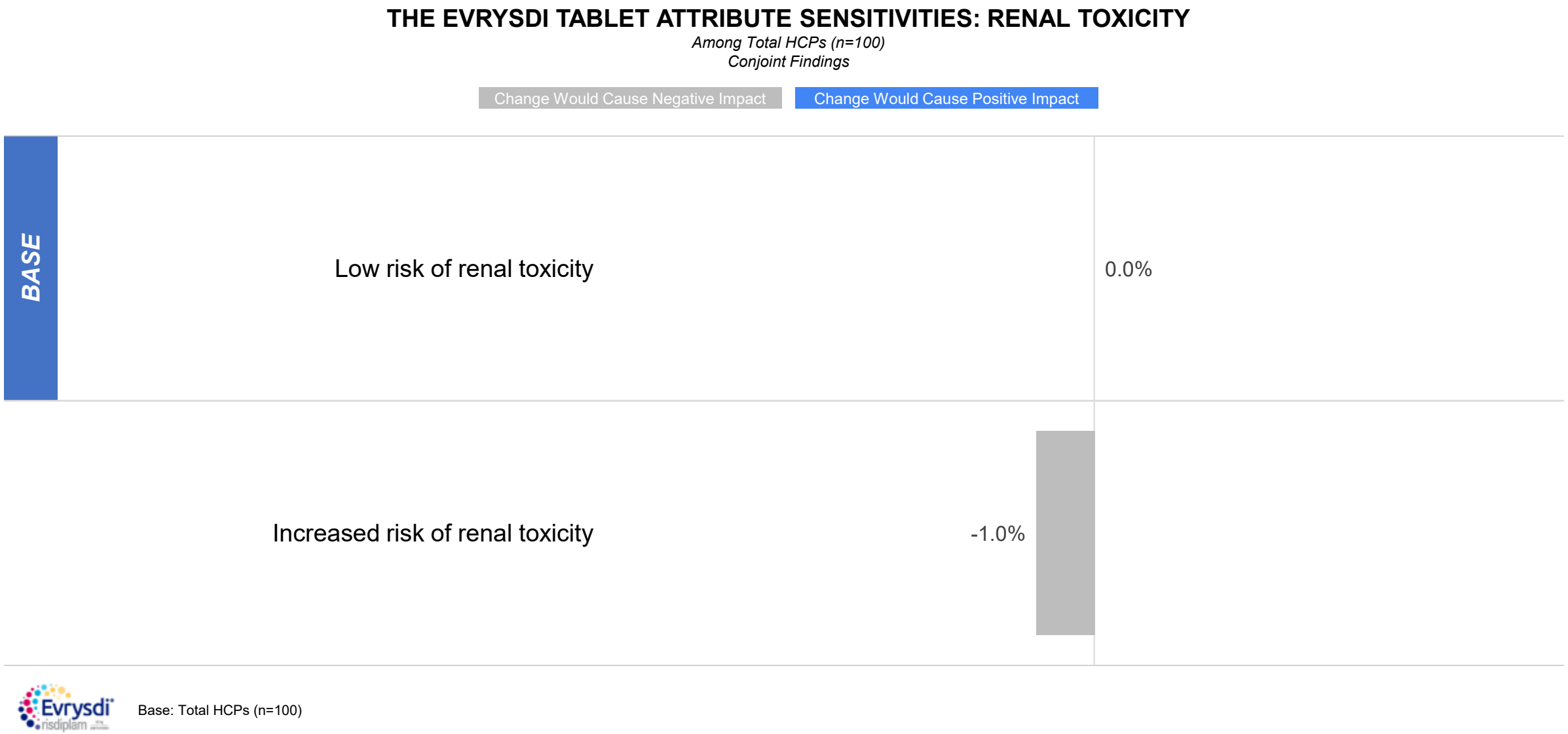
Among Total HCPs (n=100)
Conjoint Findings



Base: Total HCPs (n=100)

TABLET ATTRIBUTE SENSITIVITIES: RENAL TOXICITY

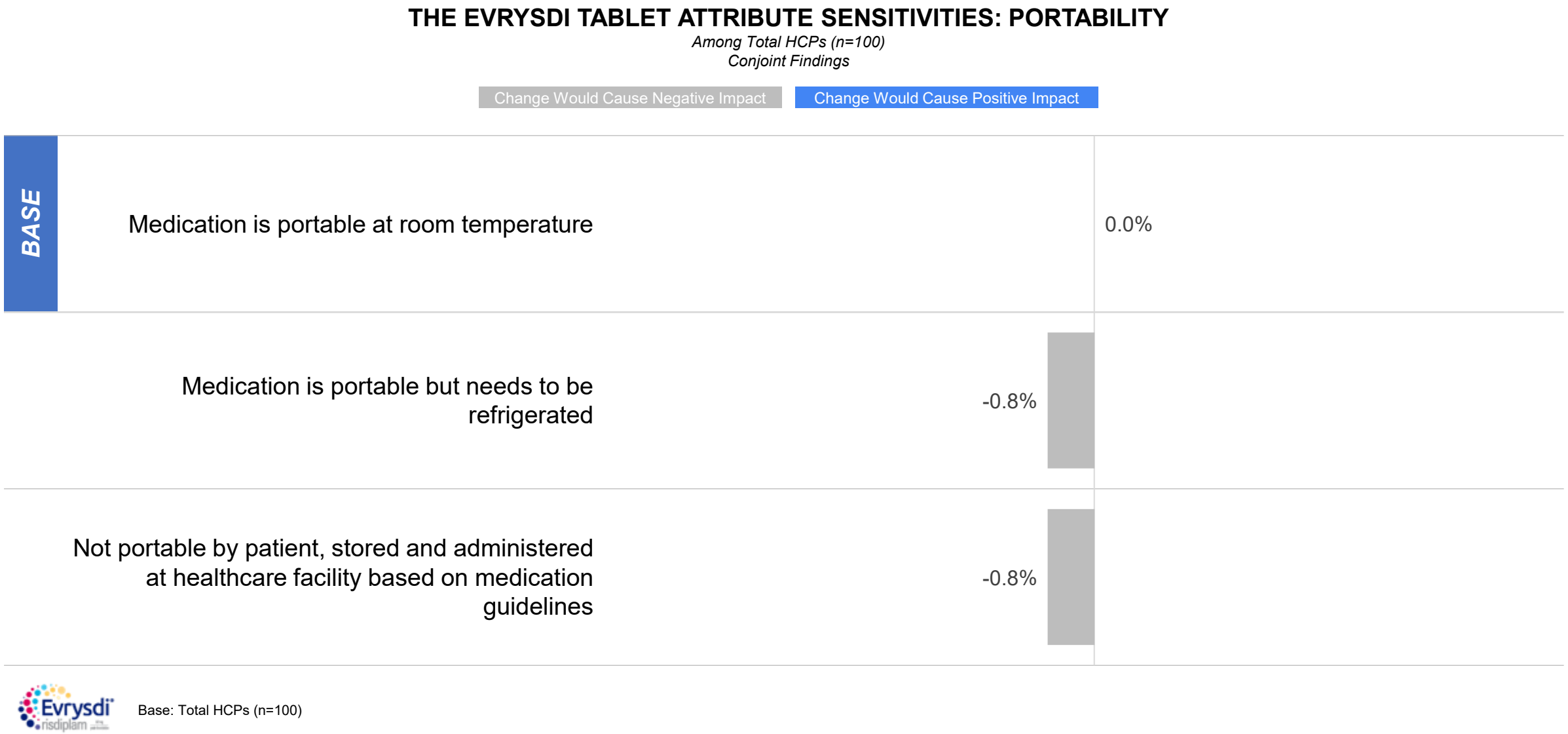
Risk of renal toxicity has a negative impact on share.



Base: Total HCPs (n=100)

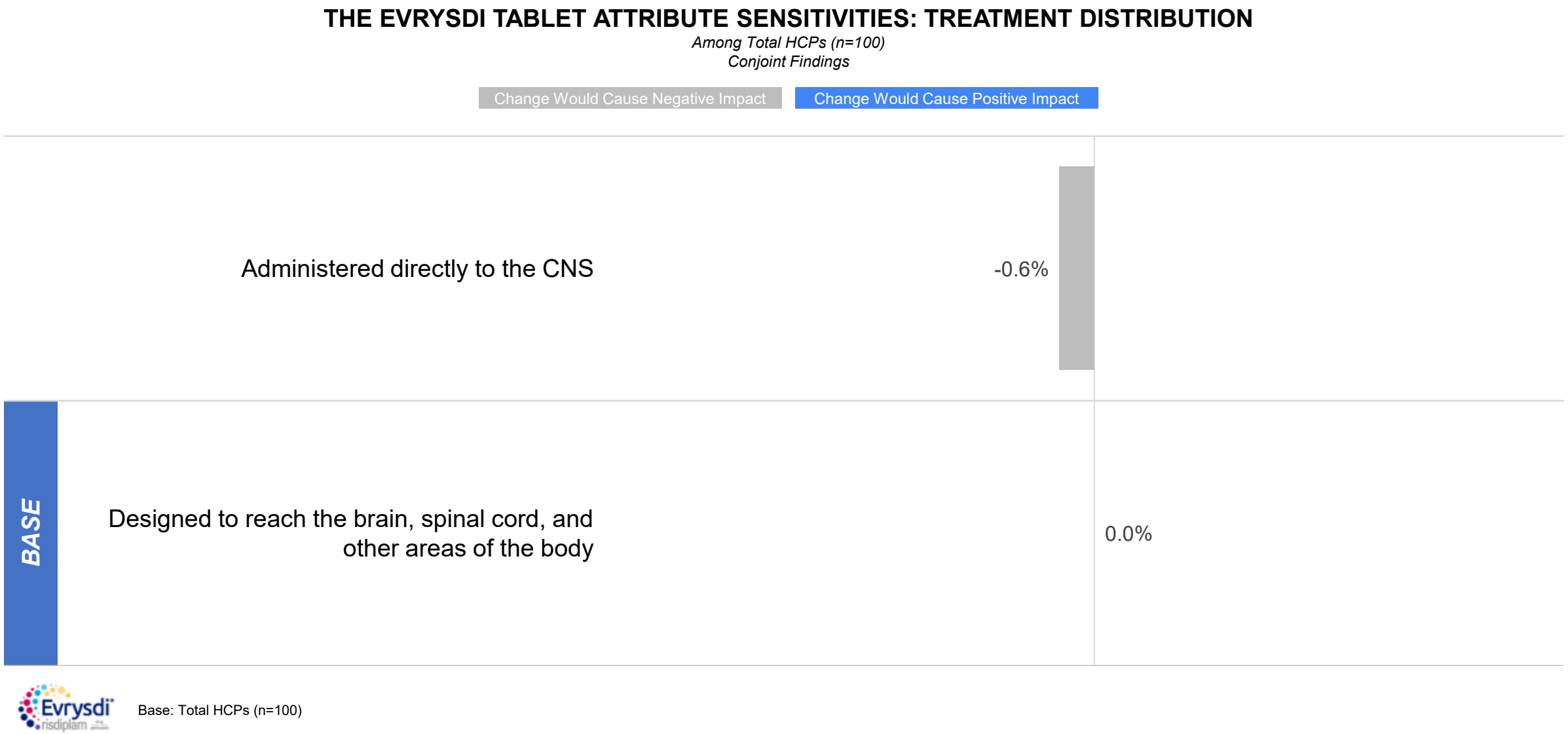
TABLET ATTRIBUTE SENSITIVITIES: PORTABILITY

Lack of portability has some impact on share.



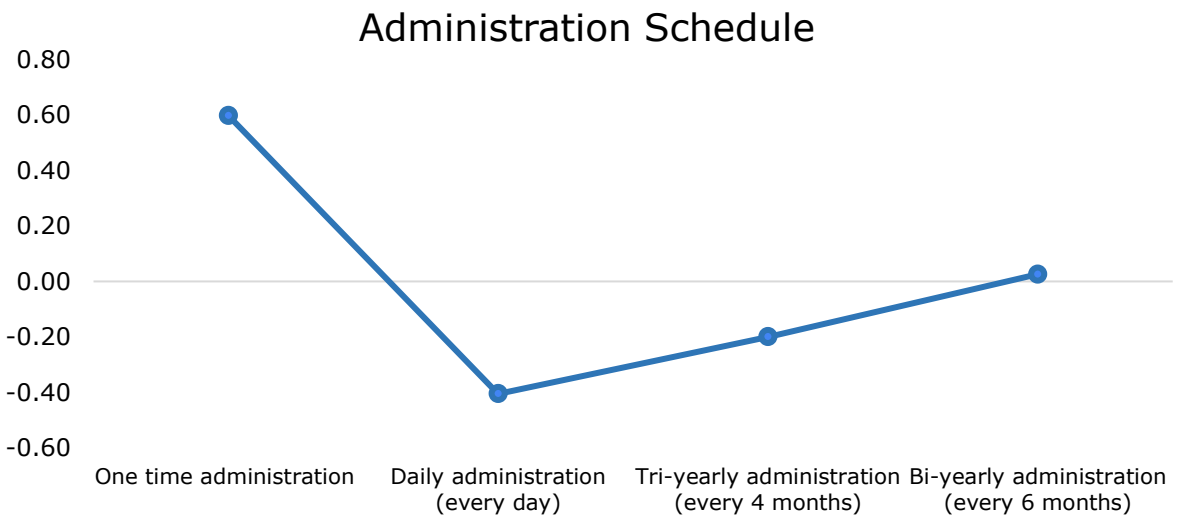
TABLET ATTRIBUTE SENSITIVITIES: TREATMENT DISTRIBUTION

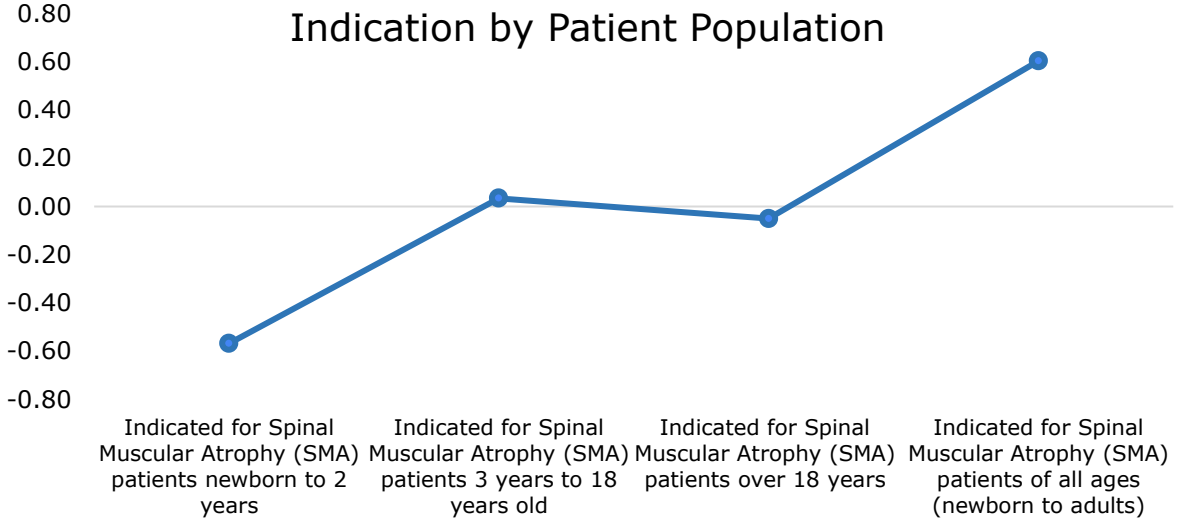
Treatment distribution has minimal impact on share.

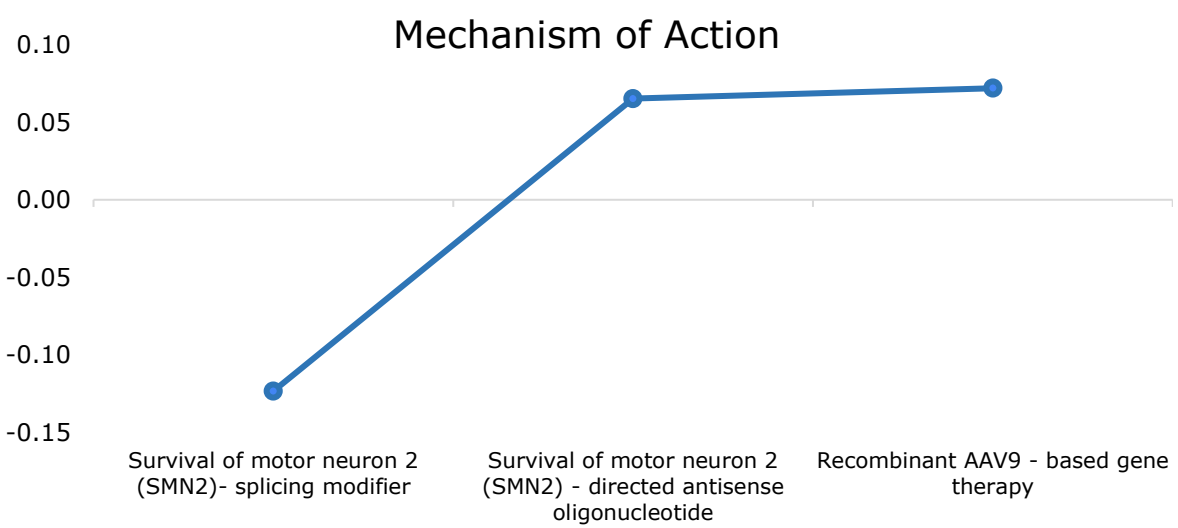


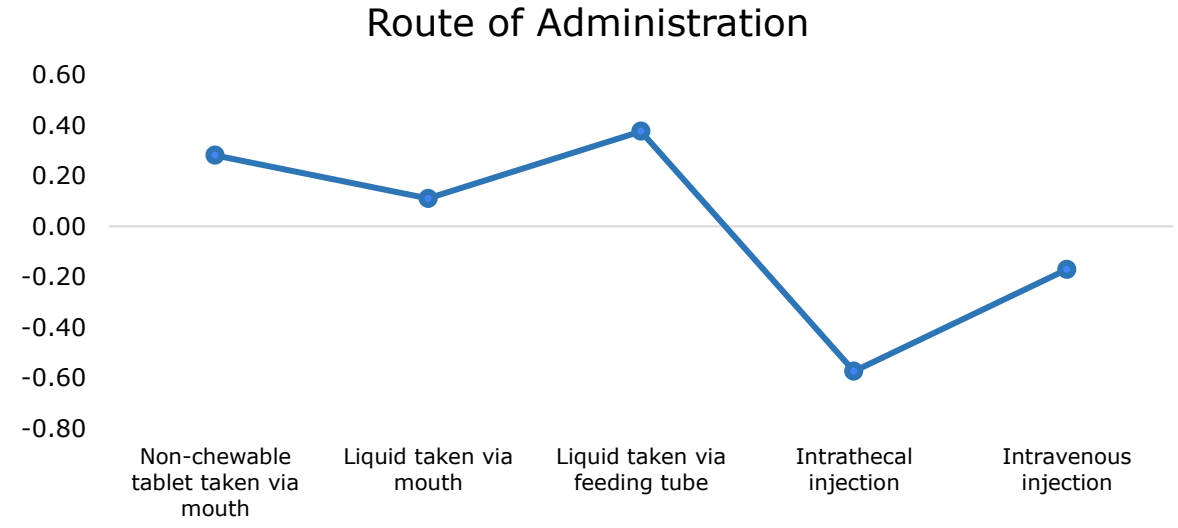
CONJOINT AVERAGE ATTRIBUTE UTILITY

Administration Schedule, Indication by Patient Population, and Route of Administration have stronger utility thus impact share.









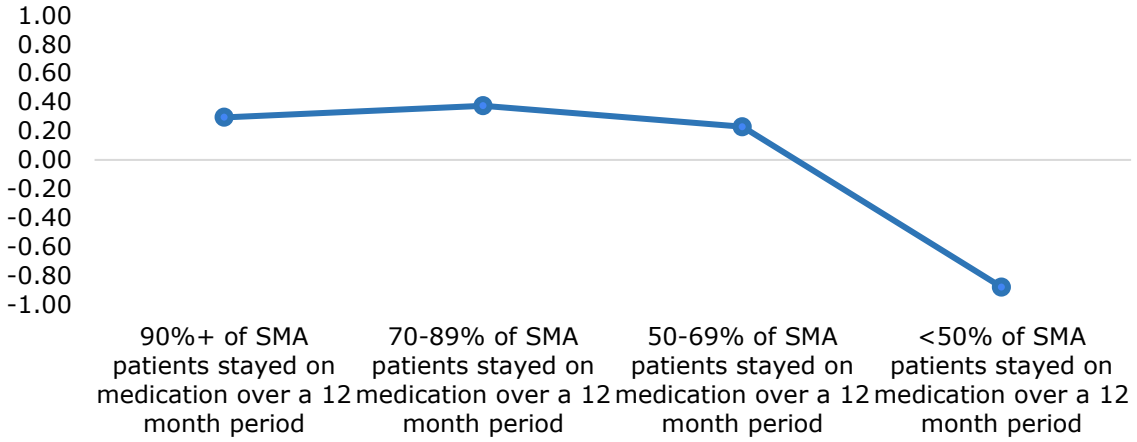
Base: Total HCPs (n=100)

Caution with Data: Output is average of all groups and preferences

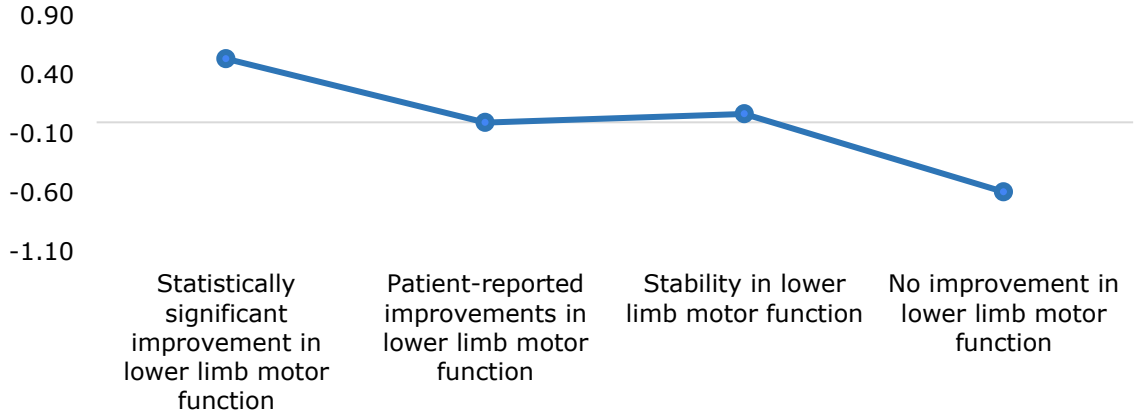
CONJOINT AVERAGE ATTRIBUTE UTILITY

Persistence, Lower Limb Motor Function, RWE, and Liver Damage have stronger utility, thus impact share.

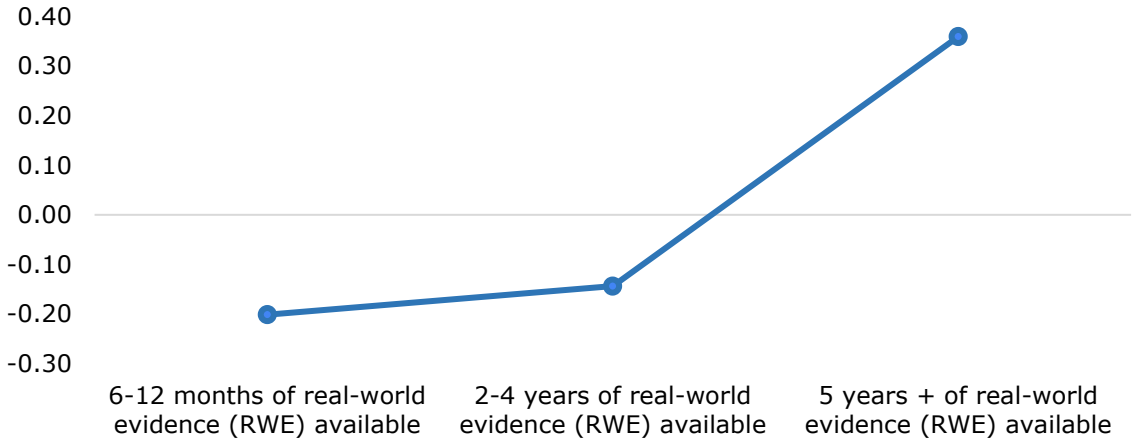
Persistence



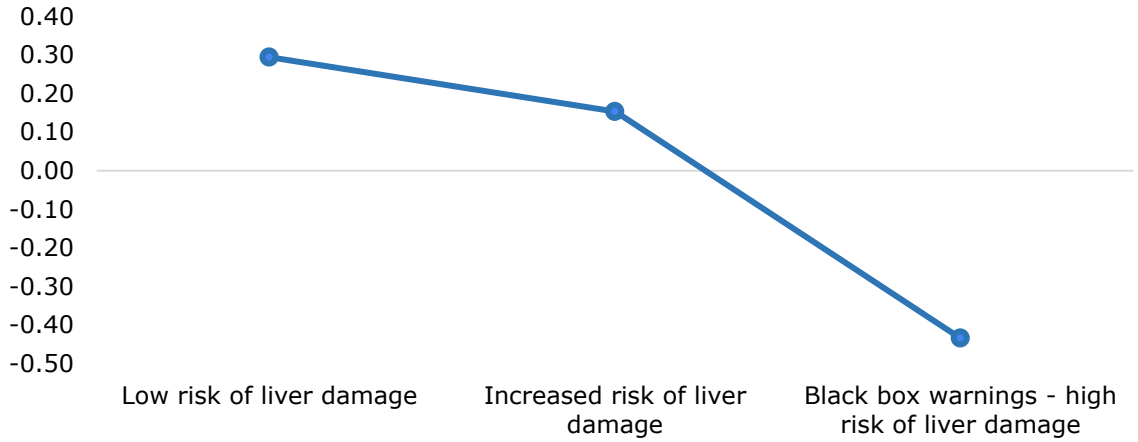
Efficacy: Lower Limb Motor Function



Real World Evidence (RWE)



Liver Damage



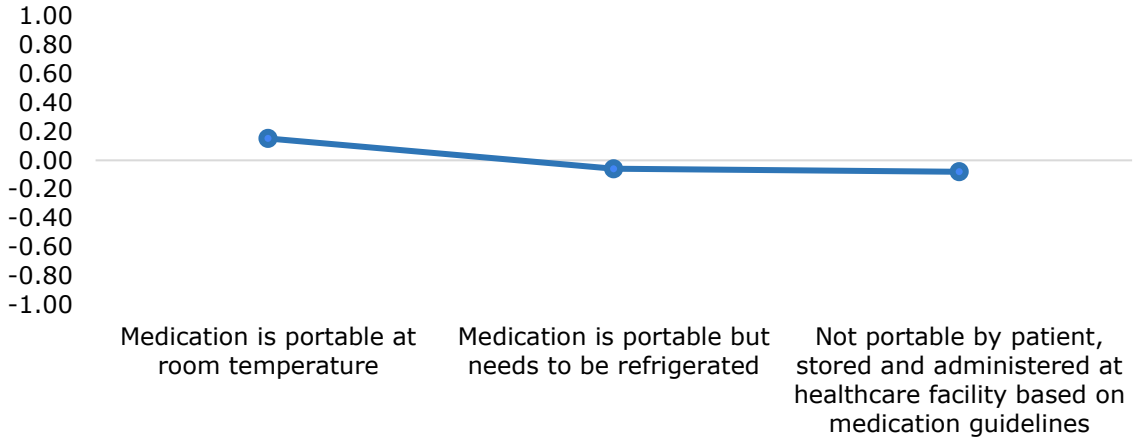
Base: Total HCPs (n=100)

Caution with Data: Output is average of all groups and preferences

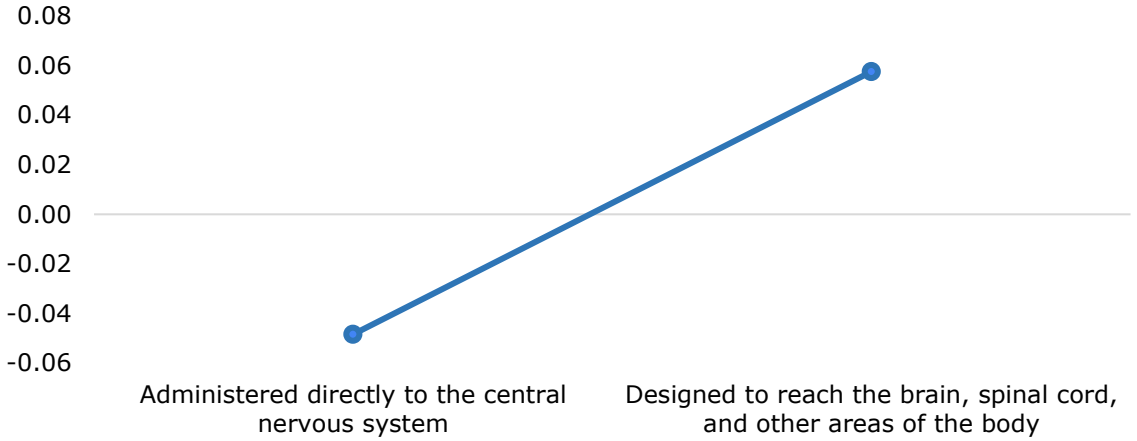
CONJOINT AVERAGE ATTRIBUTE UTILITY

Portability, Treatment Distribution, Time on Market, and Bulbar Function have lower utility, thus minimal impact on share.

Portability for Tablet

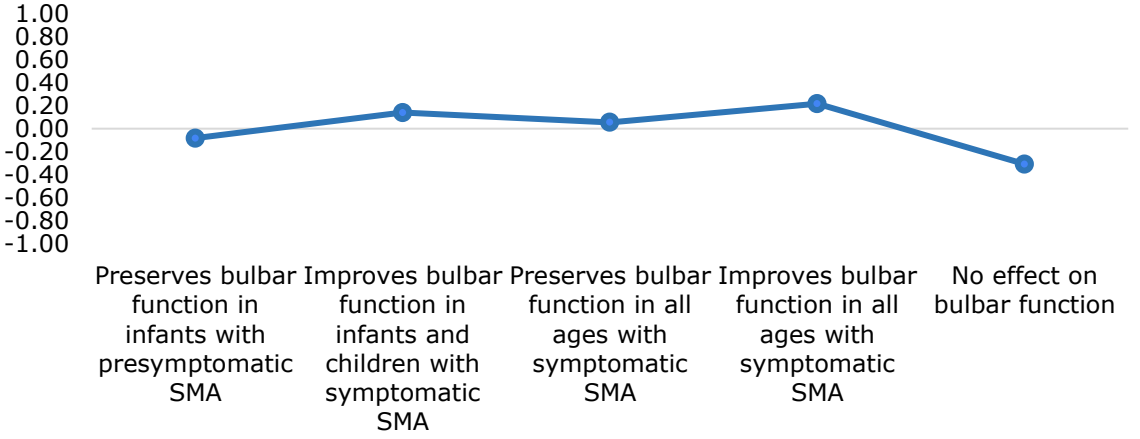


Treatment Distribution



Time on Market

Efficacy: Bulbar Function



Base: Total HCPs (n=100)

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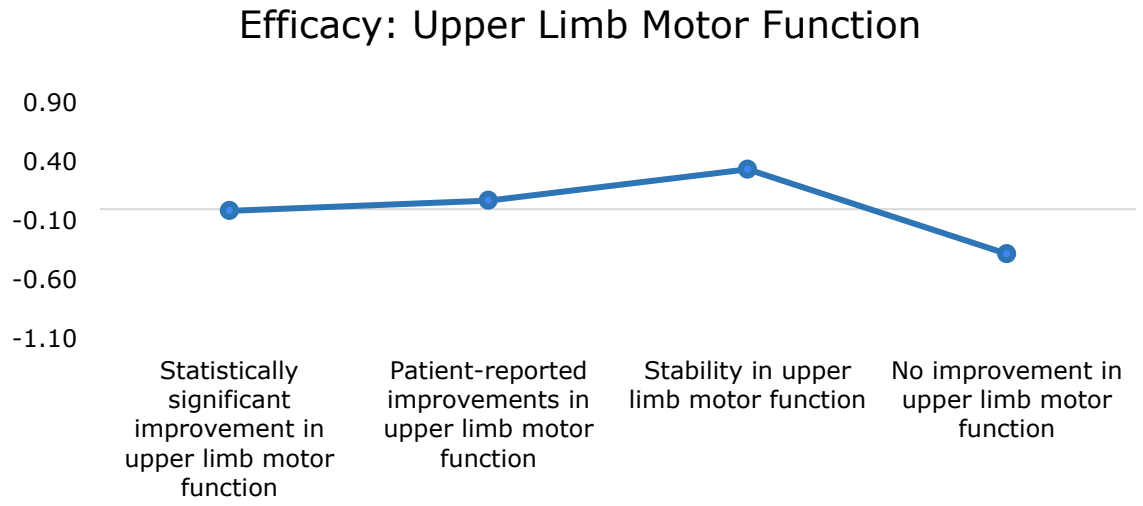
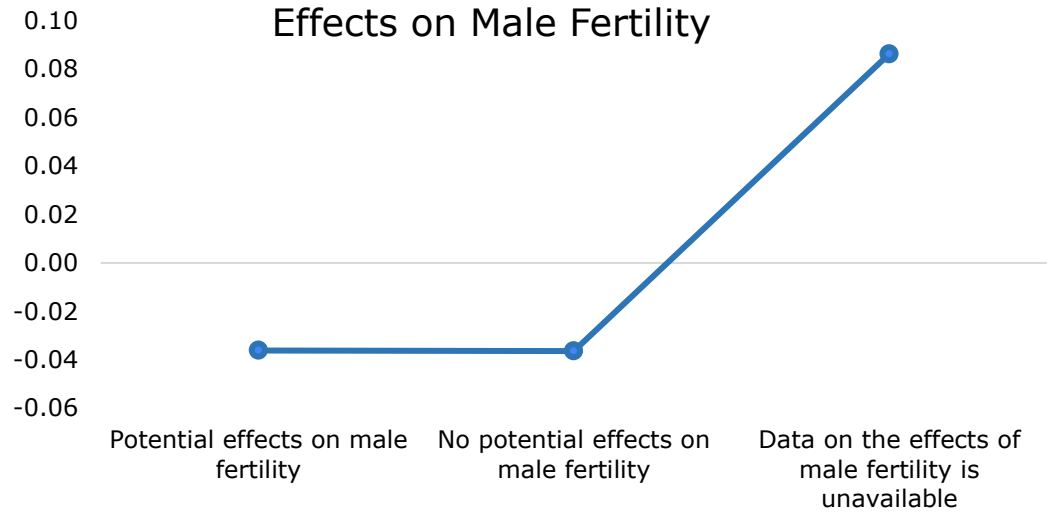
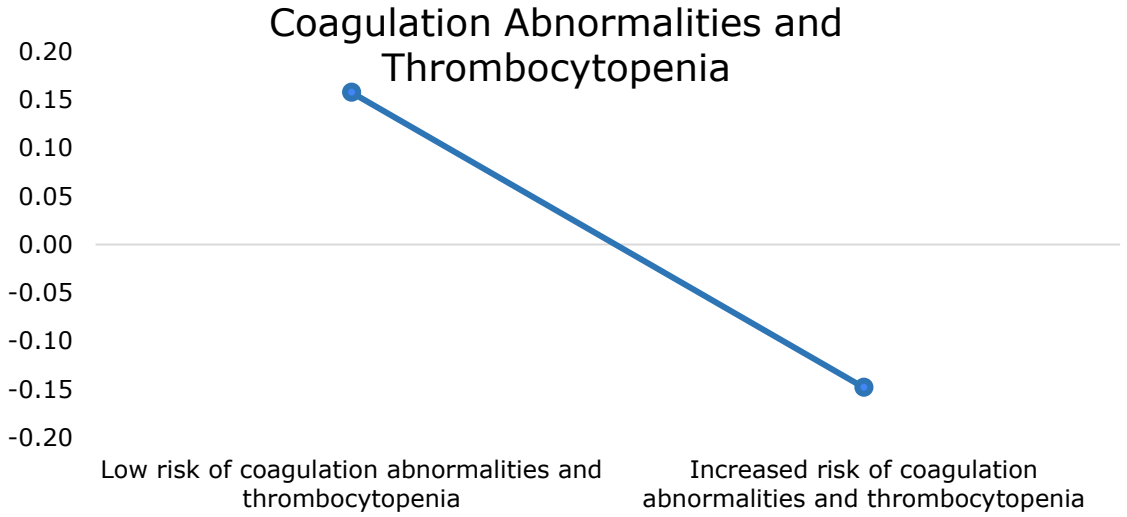
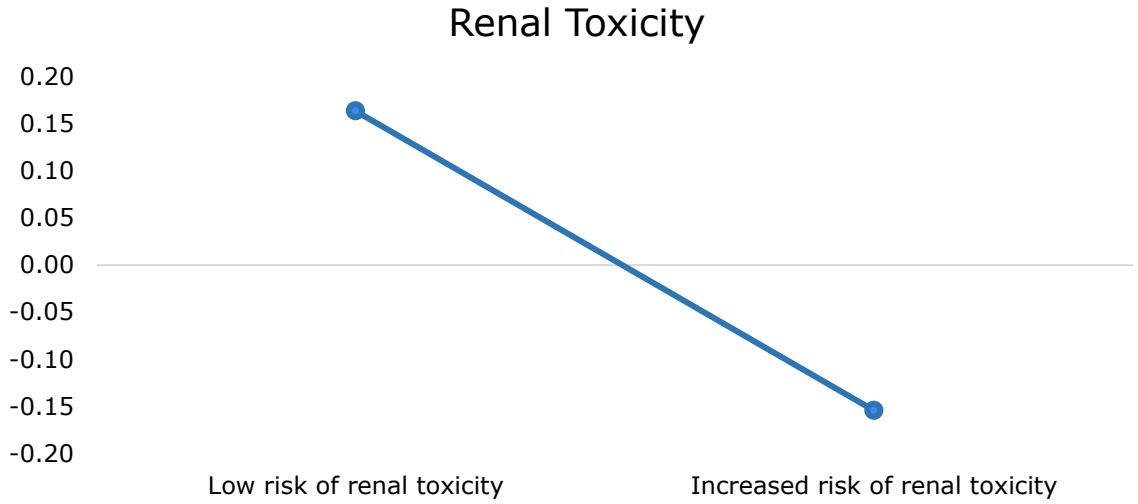
CONJOINT AVERAGE ATTRIBUTE UTILITY

Upper Limb Motor Function, Effects on Male Fertility, Coagulation Abnormalities and Thrombocytopenia, Renal Toxicity have lower utility, thus minimal impact on share.









Base: Total HCPs (n=100)

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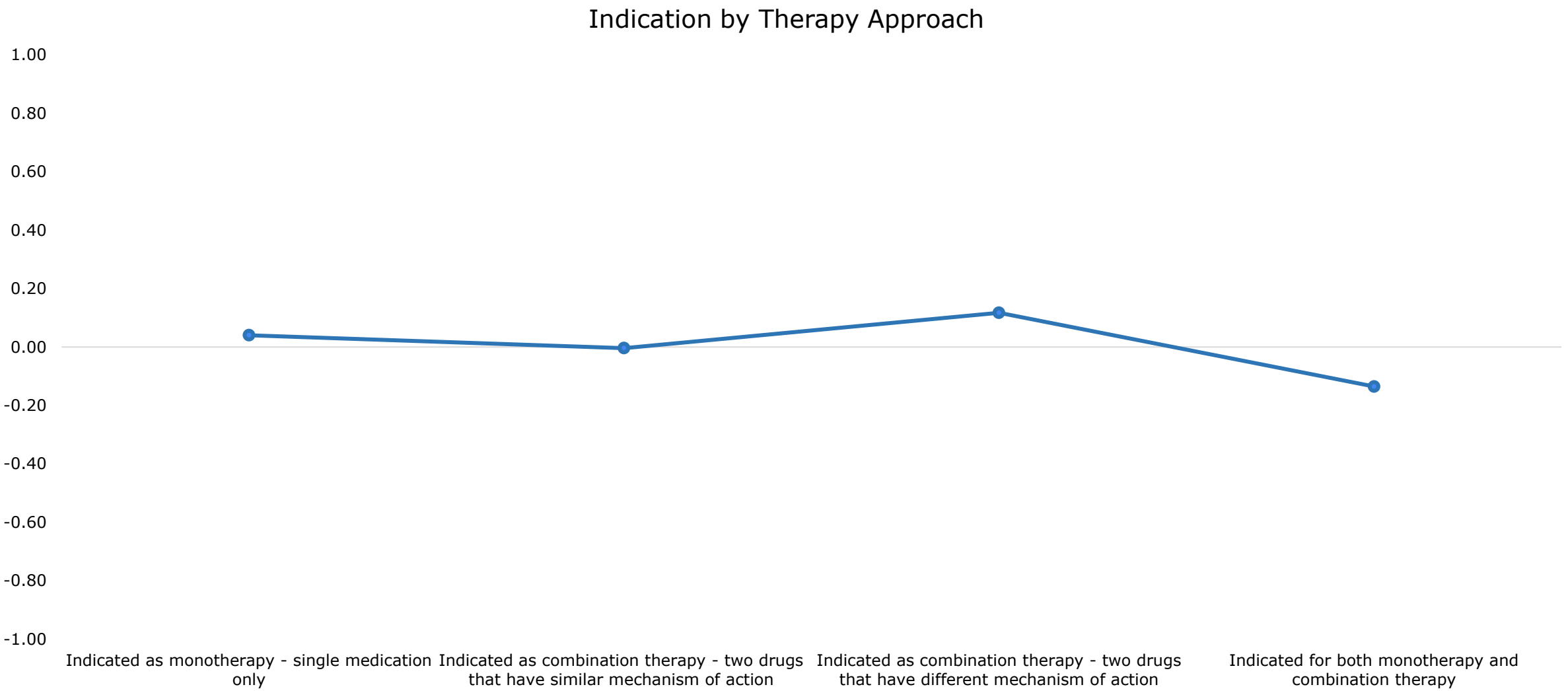
CONJOINT AVERAGE ATTRIBUTE UTILITY

Indication by Therapy Approach is low utility.



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