



PATIENTS' VIEWS ON OXBRYTA (VOXELOTOR)

Survey – 16-26 February 2021
Virtual Consultation – 18 February 2021

TABLE OF CONTENTS

1. Survey Results
2. Online Consultation
3. Summary of Patients' Views



1. SURVEY RESULTS

1. SURVEY RESULTS

- ✓ Survey run between 16-26/02/2021
- ✓ Distributed electronically to TIF's EU-based Members

Number of Participants: 27

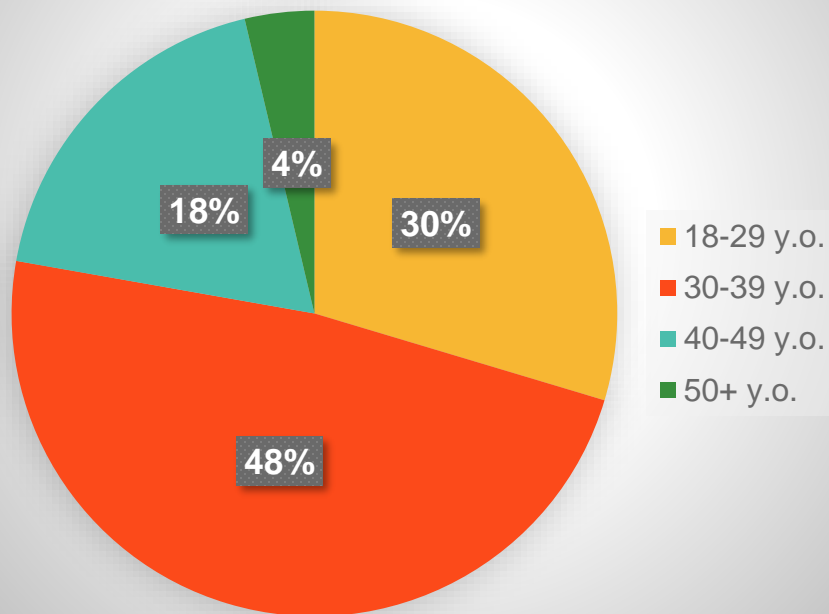
Number of Countries: 8

Austria | Cyprus | Greece | France | Germany | Ireland | Italy | Sweden

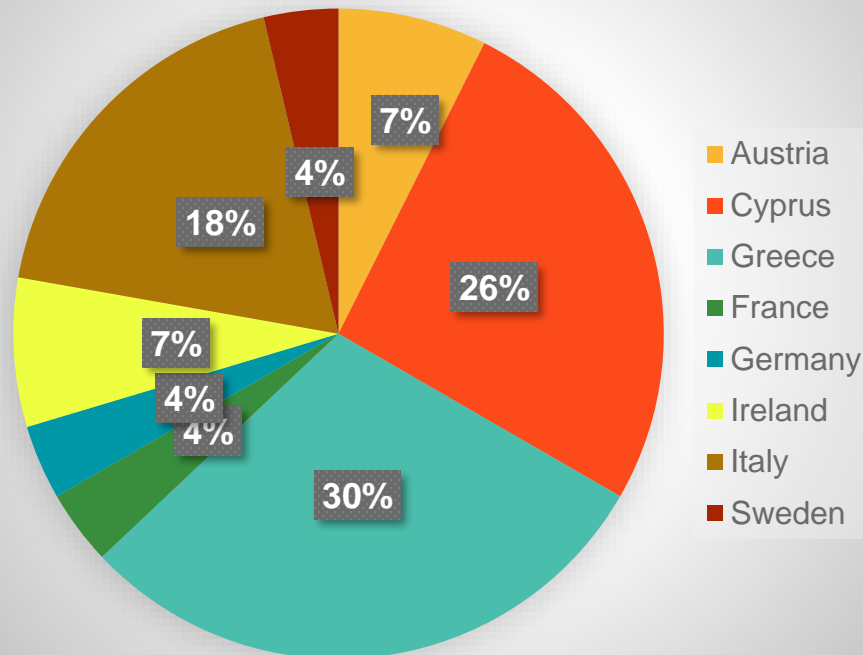
SURVEY RESULTS

Demographics

Age Distribution of Participants



Country of Residence



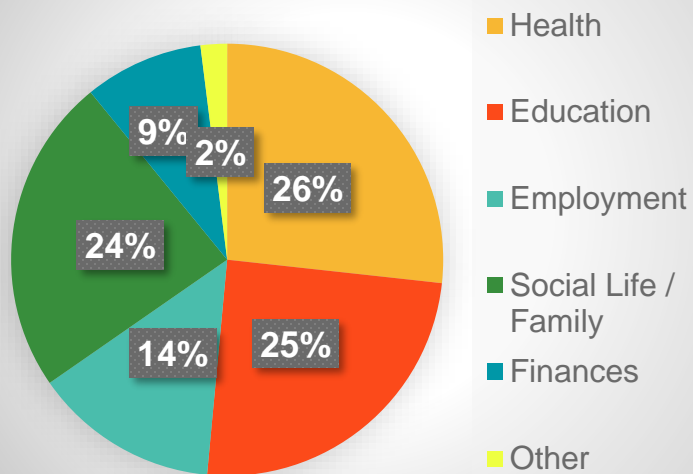
SURVEY RESULTS

Quality of Life



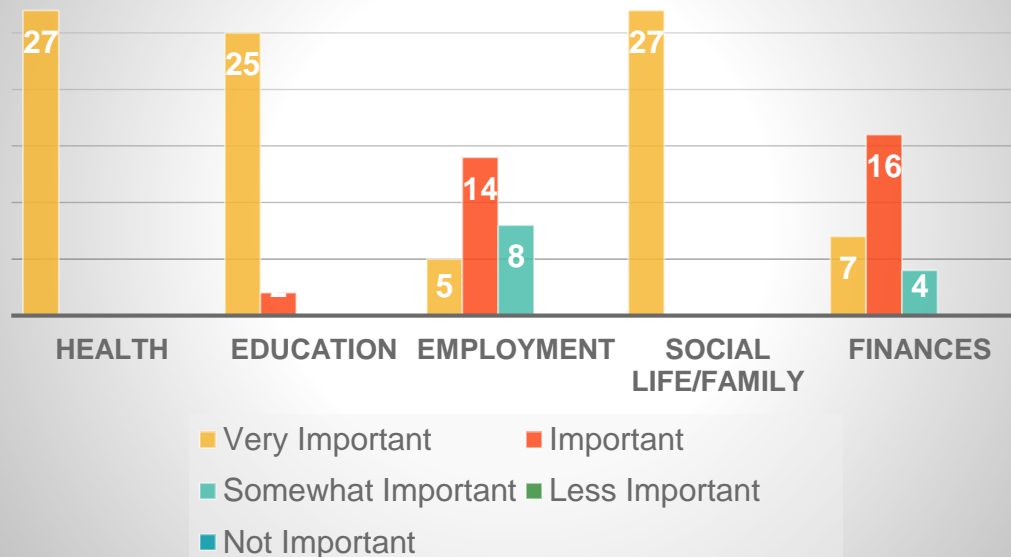
Q1: Please indicate in which of the following way(s) SCD affects your life.

Areas affected



Q2: How do you personally evaluate the importance of these aspects?

Level of Importance



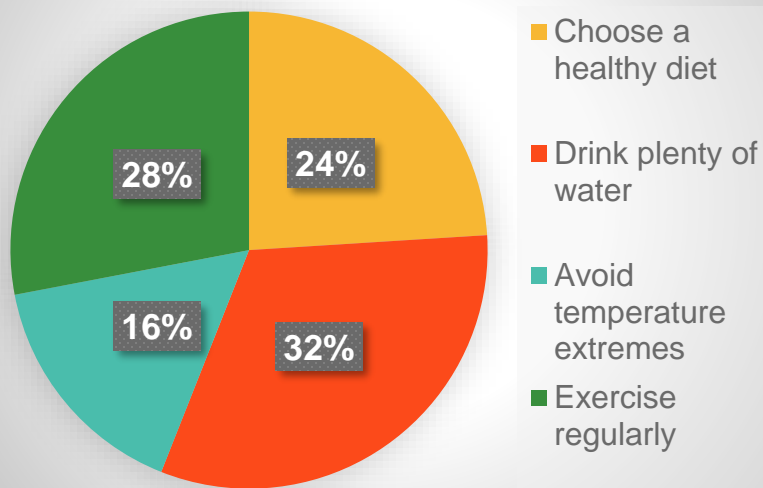
SURVEY RESULTS

Quality of Life



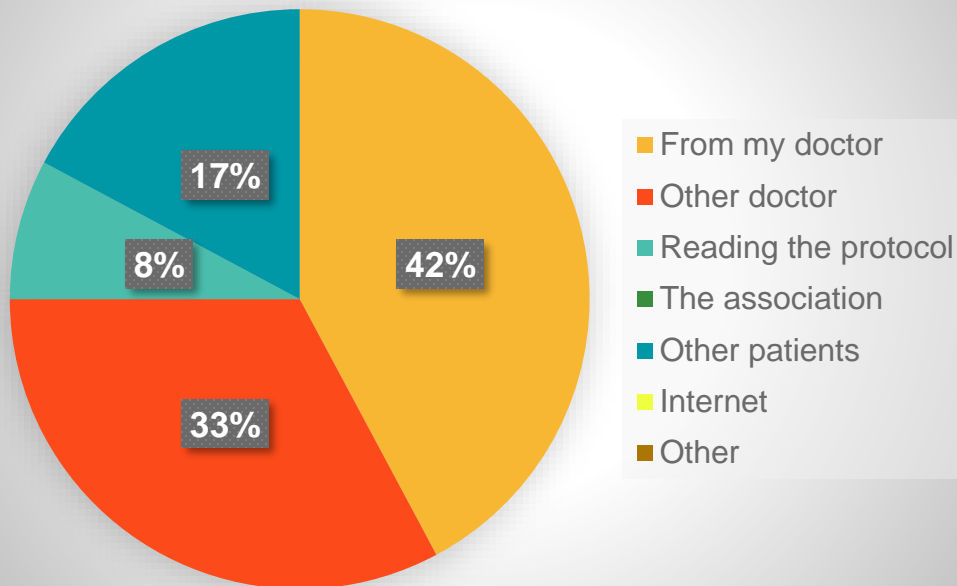
Q3: Do you...?

Lifestyle



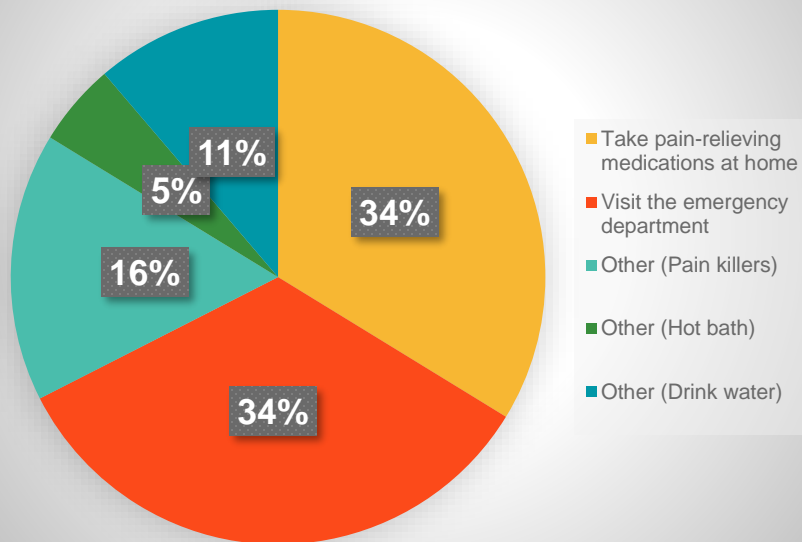
Q4: How do you find out what the correct treatment for your condition is?

Source of Information



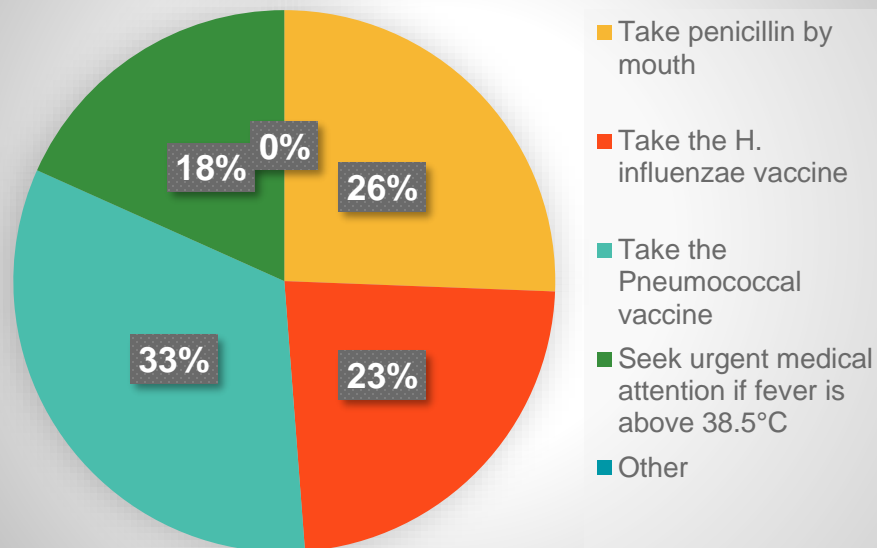
Q5: How do you usually manage pain?

Pain Management



Q6: How do you usually control infections?

Infection Management



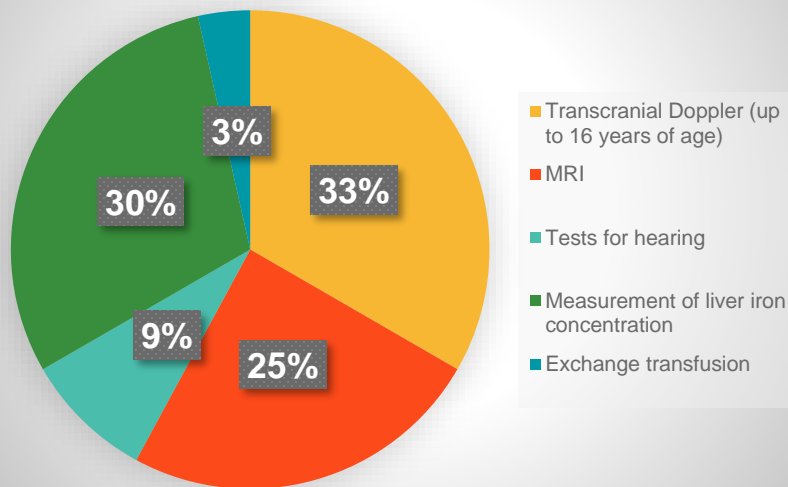
SURVEY RESULTS

Standard Treatment



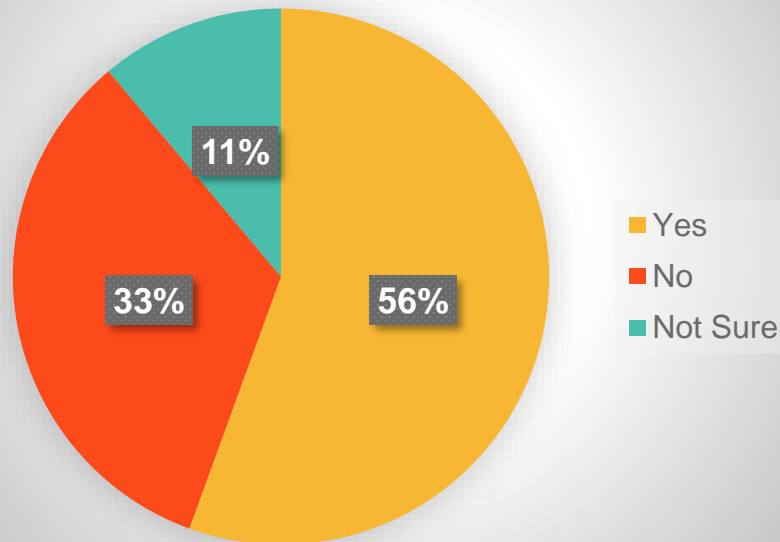
Q7: Do you have access to regular testing?

Monitoring



Q8: Do you think that the treatment that you are receiving is correct and complete?

Treatment



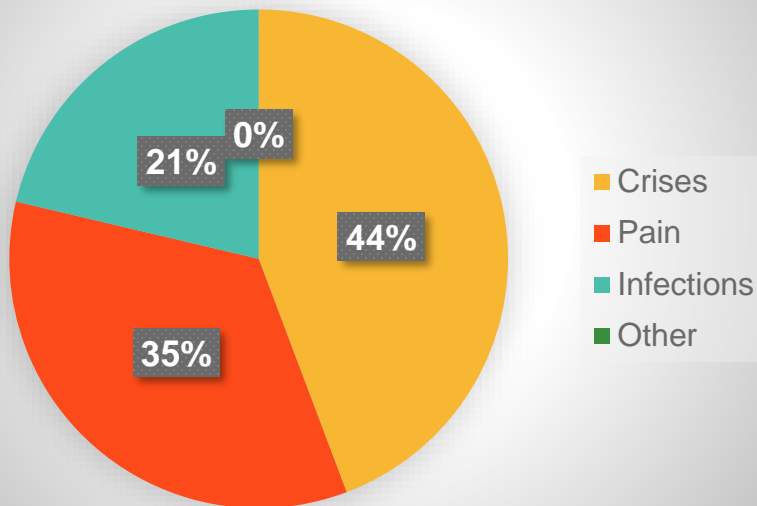
SURVEY RESULTS

Standard Treatment | New Medicines



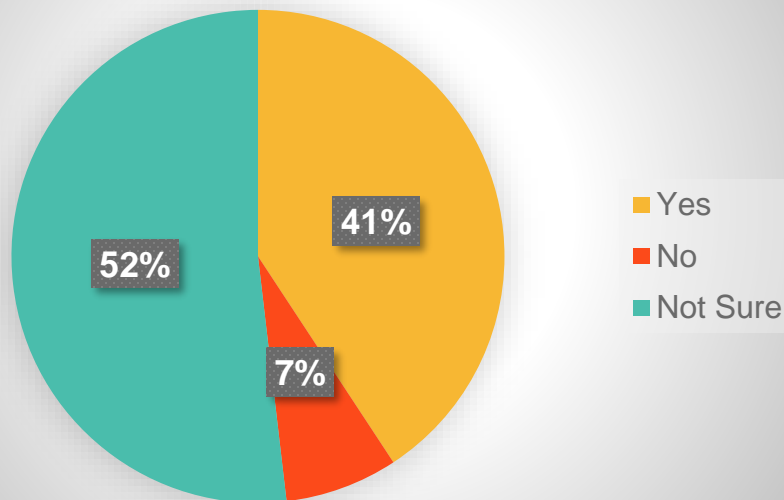
Q9: Which aspect of your condition has not been effectively addressed yet?

Unmet Needs



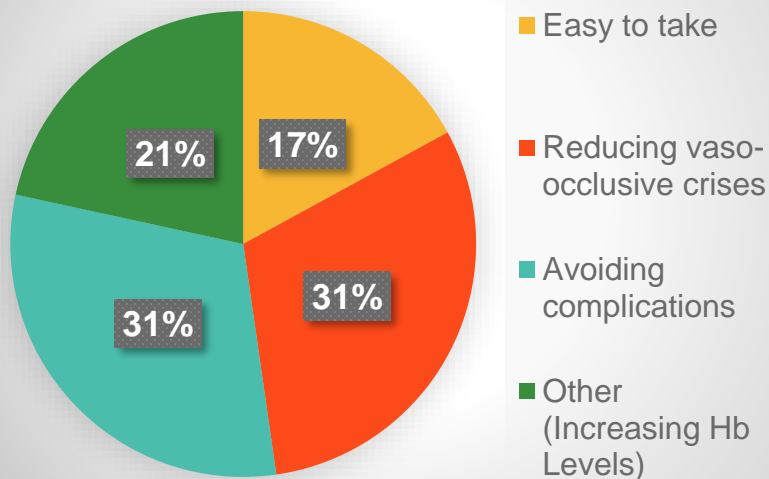
Q10: Would you accept new medicines in your treatment regime?

New Medicines



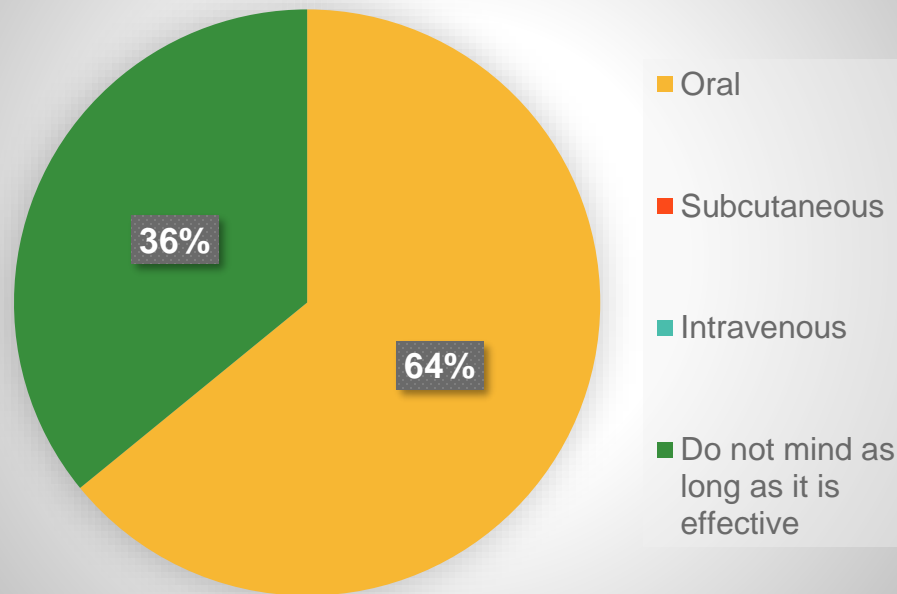
Q11: What benefits would you hope for in new medicines?

Expectations



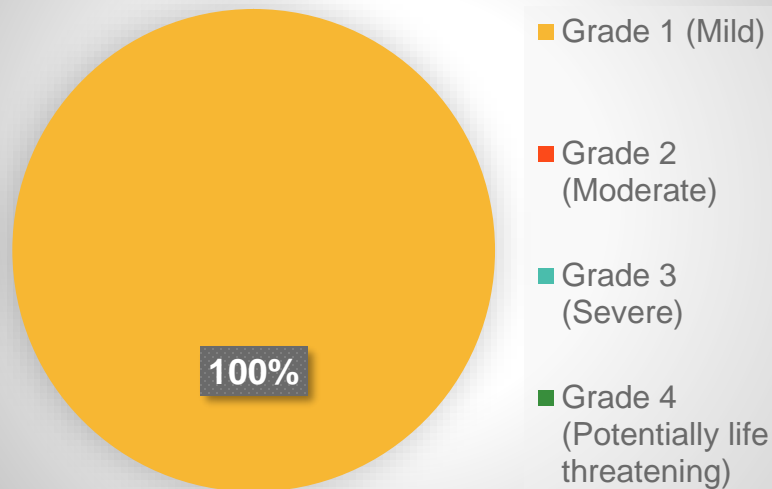
Q12: Would you prefer an oral or an intravenous treatment?

Administration



Q13: What level of side effects would you consider acceptable?

Side Effects



Q14: Are there any aspects about the condition or its treatments that you feel are not well-understood or not sufficiently considered? Please specify:

From 2 patients:

“The EMA should consider requesting additional data from countries where the Oxbryta has been marketed. This will ensure that this medicine is indeed safe to use and with minor side effects.”

2. ONLINE CONSULTATION

1. ONLINE CONSULTATION

Thursday, 18/02/2021

Number of Participants: **11**

Number of Countries: **2**

Cyprus | Greece



What is Oxbryta (Voxelotor)?

OXBRYTA is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older.

It directly inhibits sickle haemoglobin polymerization, the root cause of SCD and decreases hemoglobin S.

This effect may decrease symptoms such as tiredness, shortness of breath, and attacks of pain.

Oral
Therapy

Taken once
daily

With or
without food

Safe to take
hydroxyurea



Which
factor(s)
is/are of
concern for
you?

Do not take OXBRYTA if...



ALLERGIC

to voxelotor or any of
the ingredients in
OXBRYTA*



RECEIVING EXCHANGE TRANSFUSIONS



HAVE LIVER PROBLEMS



PREGNANT

It is not known if
OXBRYTA can harm
your unborn baby.



BREASTFEEDING

It is not known if
OXBRYTA can pass
into your breastmilk
and if it can harm your
baby.



TAKING SPECIFIC MEDICINES / HERBS

Some medicines or herbs (e.g. **St. John's wort**) may affect how
OXBRYTA works

*colloidal silicon dioxide, croscarmellose sodium, magnesium stearate, microcrystalline cellulose, and sodium lauryl sulfate. The film coating contains: polyethylene glycol 3350, polyvinyl alcohol, talc, titanium dioxide, and yellow iron oxide.

POSSIBLE SIDE EFFECTS

Common side effects

Headache |
tiredness |
diarrhoea | rash |
stomach
(abdominal) pain |
fever | nausea

Rare side effects

Serious allergic reactions

Clot in the lung |
Rush | Hives |
Shortness of breath |
Swelling of the face |
Flushing (warmth,
swelling, or redness
in your skin) |
Eosinophilia (high
counts of certain
white blood cells)

**What
do you
think?**

Do you think that
Oxbryta will affect
your **quality of life**?
To what extent?



What aspects of your
condition have **not**
been **addressed yet**
(e.g. infections, pain
management, crises)?



What level of **side effects** would you
consider acceptable?



Levels of side effects

GRADE 1 (Mild)

Transient (goes away after a short time) or mild discomfort; no limitation in activity; no medical intervention/therapy required.

GRADE 2 (Moderate)

Your daily activity is affected mild to moderately – some assistance might be needed; no or minimal medical intervention/therapy required.

GRADE 3 (Severe)

Your daily activity is markedly reduced – some assistance usually required; medical intervention/therapy required, hospitalisation or hospice care possible.

GRADE 4 (Potentially life threatening)

Extreme limitation to daily activity, significant assistance required; significant medical intervention/therapy, hospitalisation or hospice care very likely.

Is there anything else you
want the EMA to know
and take into account
while evaluating Oxbryta?



Feedback received

- ✓ Adverse events should not interfere with the patients' **well-being** nor be present on a permanent basis.
- ✓ **More information** is needed regarding the "rare" side effects, maybe from the US where 2,000 people already use Oxbryta.
- ✓ Only **Level 1 Side Effects** can be tolerated. More severe side effects are not accepted by any patient.
- ✓ **Pain management** is still an unaddressed need.
- ✓ The **increase of Hb** is important as many patients experience severe haemolysis and are transfusion-dependent.
- ✓ It seems that the only benefit of Oxbryta (compared to hydroxyurea that has no side effects) is the **slight increase of the haemoglobin** – this should be further investigated.

3. SUMMARY OF PATIENTS' VIEWS

Summary of Patients' Views

1. People with SCD believe that their condition directly and mostly affects their **social life and family**. What they need and want is a constant feeling of **well-being**. This is an unmet need, as the existing treatments do not effectively address OVC crises and do not render patients transfusion-independent, increasing their haemoglobin levels.
2. Awareness regarding **lifestyle** best practices varies between participants, while the only reliable source of information is each patients' **treating physician**. Patients still take pain-relieving medications at home and visit the **emergency department** in case of crisis. This for further training of HCPs on the clinical management of the condition to ensure that everyone receives quality care.

Summary of Patients' Views

3. Most patients do not have access to appropriate and regular **testing**. This renders monitoring rather difficult for their treating physicians. Most importantly, a great number of patients does not think that **existing treatment** is correct nor complete.
4. People with SCD expect a treatment that will help them address/better manage **crises**. They are open to new interventions, as long as adequate information is provided regarding **side effects** and how these may affect their daily lives.
5. Most patients are in favour of **oral** treatments.