Review of Institute for Clinical and Economic Review (ICER) Reports: Use and Accessibility of Surveys to Incorporate the Patient Perspective

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UNIVERSITY of MARYLAND BALTIMORE

Background and Problem Statement

- Value assessment (VA) is a tool increasingly used in the United States (similar to HTA outside the US) to aid healthcare stakeholders when estimating the value of treatments.[1-6]
- V/HTAs have been criticized for lacking patient centeredness.[7-11]
- Recent and continuing efforts are being made to advance patient centricity in V/HTA.[12-17]
- Patient-experience survey data collected by *patient organizations* can be leveraged to inform patient-centered V/HTA.

While patient engagement in V/HTA has increased over time, it is unknown if use of patient-group survey data in V/HTA reports has increased as well

U.S. Value Assessment Tools

	ICER Evidence Reports	IVI Open-Source Value Project Models	NCCN Evidence Blocks (EBs)
STRUCTURE			
Target audiences	Payers and policymakers	Patients, providers, policymakers, manufacturers, and payers	Providers and patients
Services assessed	Primarily pharmaceutical treatments, some health care services	Primarily pharmaceutical treatments	Pharmaceutical treatments
Conditions assessed	Any condition	Any condition	Oncology
Assessment output	Comparative cost-effectiveness estimates / Health-benefit price benchmark / Budget impact estimates	Customizable comparative cost-effectiveness estimates / Customizable assessment of overall value based on MCDA	EB scores for five domains: efficacy, safety, quality of evidence, consistency of evidence, affordability
ASSESSMENT PROCESS			
Number of assessments*	65 completed reviews** / 4 in-process reviews**	2 completed models / 1 in-process model	EBs included in 61 NCCN Guidelines
Public comment	Opportunity for public comment on draft scoping / document and draft evidence report / Public meeting allows time-limited invited comments	Opportunity for public comment on model scope, model protocol, and following each model release	No public comment period in the development of EBs / Stakeholders can submit data and request a change after an EB is published
Patient engagement	Includes patient stakeholders in its assessment process	Includes patient stakeholders in its model planning and development processes	None

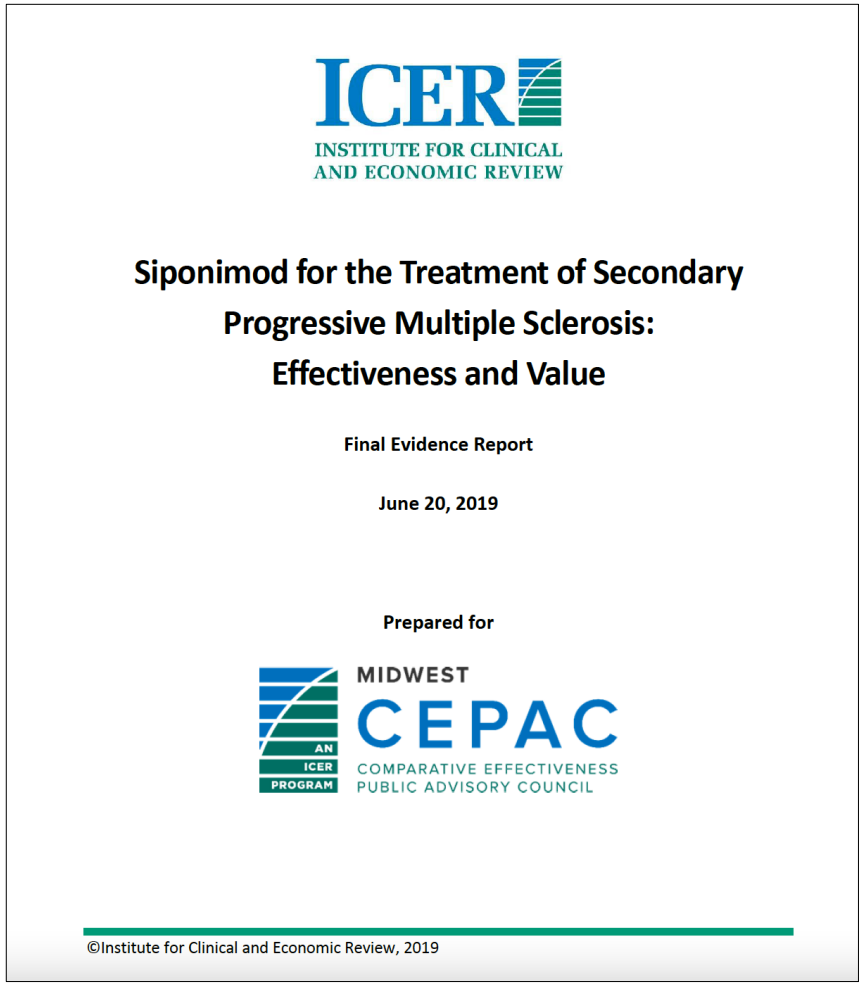
* Number of assessments as of December 2022. ** Topic reviews counted since implementation of ICER Value Assessment Framework.
Source: https://www.phrmafoundation.org/wp-content/uploads/2023/01/Driving-Progress-in-U.S.-Value-Assessment_PhRMA-Foundation.pdf

Objective and Methods

Objective: Describe use of patient-experience surveys in Institute for Clinical and Economic Review (ICER) reports.

- **Study Design:** Exploratory, descriptive study
- **Study Sample:** ICER final evidence reports
- **Data Sources:** ICER and patient group websites
- **Inclusion Criteria:**
  - ICER final reports from 2017-2022
  - Publicly available
- **Descriptive Attributes for Abstraction:**
  - Condition
  - Report date
  - Report title
  - Survey data from patient group
  - Survey publicly available
  - Report update
  - Condition revisited

Search Strategy for Patient Experience Input via Surveys



ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

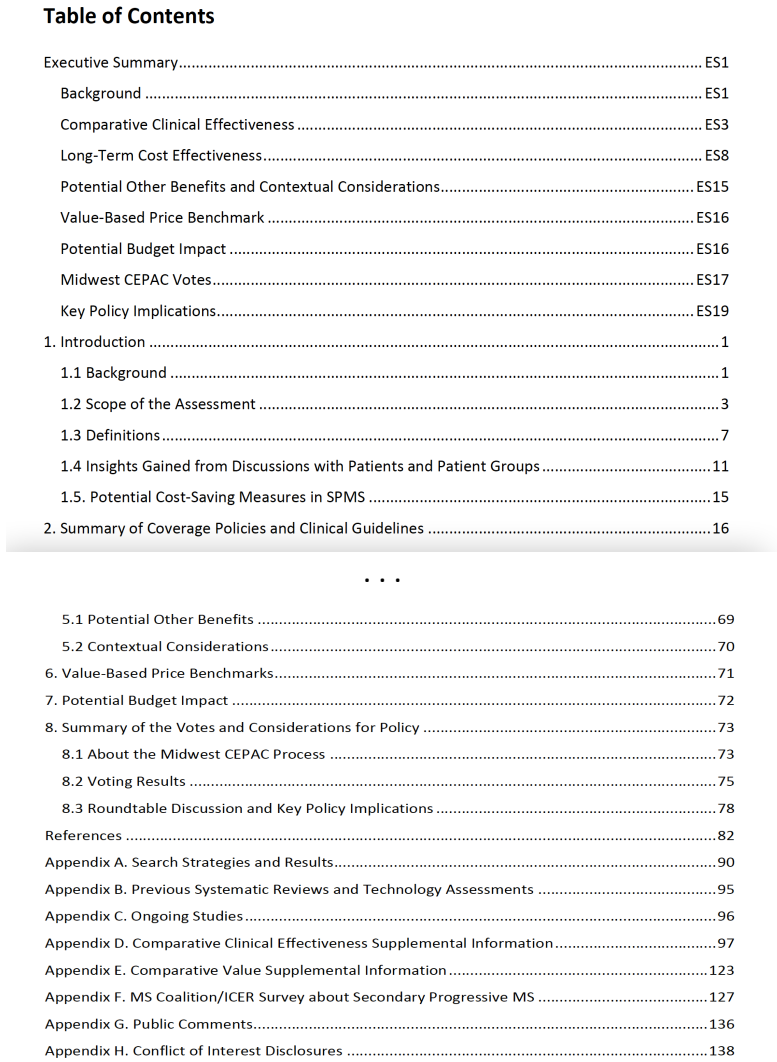
Siponimod for the Treatment of Secondary Progressive Multiple Sclerosis: Effectiveness and Value

Final Evidence Report

June 20, 2019

Prepared for

MIDWEST CEPAC
AN ICER PROGRAM
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL



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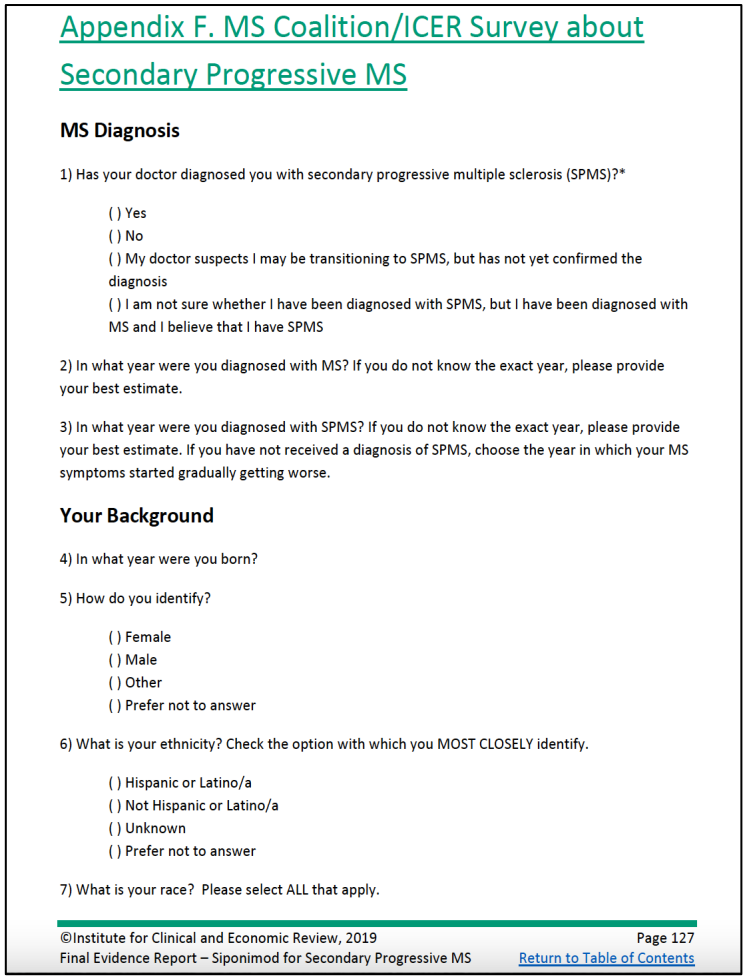

Appendix F. MS Coalition/ICER Survey about Secondary Progressive MS

MS Diagnosis

1) Has your doctor diagnosed you with secondary progressive multiple sclerosis (SPMS)?*

( ) Yes
( ) No
( ) My doctor suspects I may be transitioning to SPMS, but has not yet confirmed the diagnosis
( ) I am not sure whether I have been diagnosed with SPMS, but I have been diagnosed with MS and I believe that I have SPMS

2) In what year were you diagnosed with MS? If you do not know the exact year, please provide your best estimate.

3) In what year were you diagnosed with SPMS? If you do not know the exact year, please provide your best estimate. If you have not received a diagnosis of SPMS, choose the year in which your MS symptoms started gradually getting worse.

Your Background

4) In what year were you born?

5) How do you identify?

( ) Female
( ) Male
( ) Other
( ) Prefer not to answer

6) What is your ethnicity? Check the option with which you MOST CLOSELY identify.

( ) Hispanic or Latino/a
( ) Not Hispanic or Latino/a
( ) Unknown
( ) Prefer not to answer

7) What is your race? Please select ALL that apply.

Source: https://icer.org/wp-content/uploads/2020/10/ICER_MS_Final_Evidence_Report_062019.pdf

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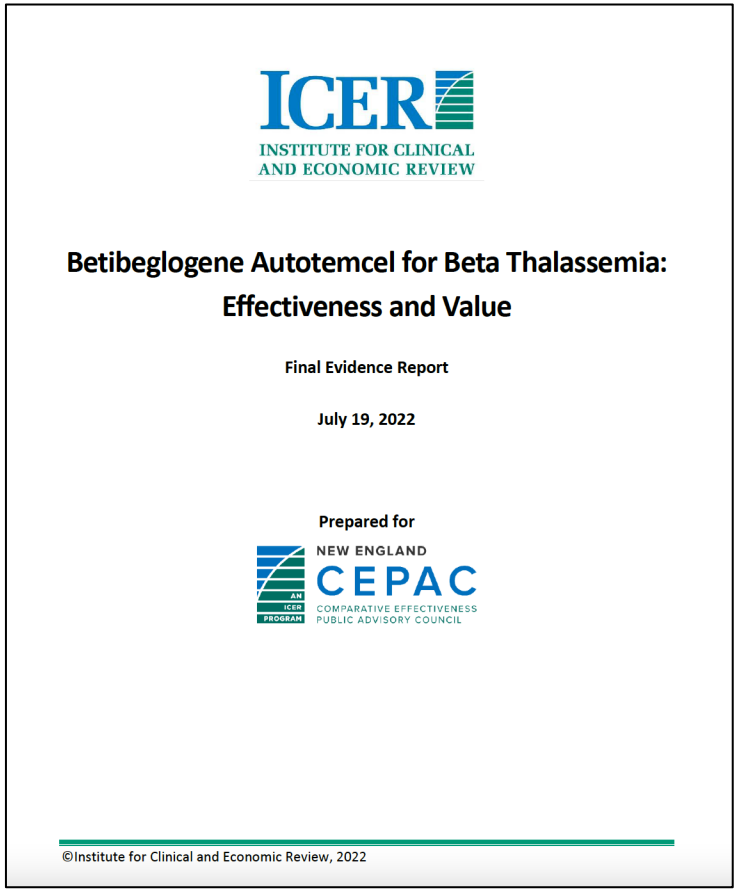
Case Study #1: Beta Thalassemia

Survey Available



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Betibeglogene Autotemcel for Beta Thalassemia: Effectiveness and Value

Final Evidence Report

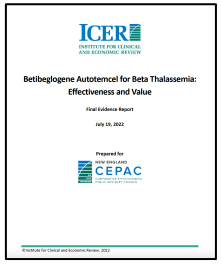
July 19, 2022

Prepared for

NEW ENGLAND CEPAC
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL

Source: https://icer.org/wp-content/uploads/2021/11/ICER_Beta-Thalassemia_Final-Report_071922.pdf

Case Study #1: Beta Thalassemia - Survey Available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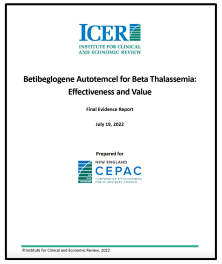
2. Patient and Caregiver Perspectives

From 2011 to 2021 the median age of death for a person in the US with TDT was 37.[3] Although life expectancy is likely to continue to increase with improved treatments, it still lags behind population norms. Additionally, patients with TDT still report decreased quality of life due to the impact on physical and mental health.[4,5] Patients and clinicians reported that living with severe forms of beta thalassemia requires being "tethered to the health care system" and often to a geographic area with a medical center that can provide thalassemia care. Management of the condition has been described as a part-time job for patients and their caregivers. Patients plan their lives around transfusions, disrupting their ability to travel, take vacations, work, and study. Some patients receive transfusions as often as every two weeks, in addition to regular MRI and DEXA imaging, monitoring of laboratory values, and visits to clinical specialists (e.g., endocrinologists, cardiologists). Regular transfusions and chelation can be technically challenging in young children, causing stress in patients and caregivers. Some patients require semi-permanent catheters (i.e., ports) to facilitate regular transfusions, which carry a risk of infection. Patients and their caregivers also spend hours managing administrative aspects of their condition, such as calling doctors' offices and navigating insurance policies. Patients and caregivers emphasized how quality of life is also markedly impacted by other manifestations of thalassemia (e.g., diabetes, problems with growth or development). Infertility, including as a consequence of HSCT, was highlighted as a particular concern among all groups of stakeholders, including adult patients, caregivers of children, and clinicians. . . .

. . . We met with representatives from the Thalassaemia International Federation (TIF), the largest global thalassemia advocacy group, about the perspective of patients and caregivers. In a 2020 report by TIF, the majority of survey respondents with thalassemia demonstrated a positive attitude towards gene therapy with a significant proportion interested in gene therapy for themselves or their children. In developed countries (including the US), however, 29% of patients reported that they would accept gene therapy, while the remaining patients reported they would not accept gene therapy (24%) or were uncertain (47.2%). This mirrors what we heard from patients in our stakeholder interviews. While there was general enthusiasm toward the possibility of curing beta thalassemia, some patients whose condition is well-managed with current standard of care thought they might be reluctant to modify their treatment regimen because beti-cel has only been evaluated in small numbers of patients and treatment requires chemotherapy and hospitalization. Other adults we spoke with expressed a strong interest in gene therapy and thought they would pursue it if eligible. We spoke with caregivers of adult children living with TDT and also parents of a child who had undergone a sibling matched HSCT. These caregivers thought there would be a strong interest in gene therapy among parents with young children. When probed about what a cure for TDT would mean, one participant told us "Time… and you just can't put a price on that."

Source: https://icer.org/wp-content/uploads/2021/11/ICER_Beta-Thalassemia_Final-Report_071922.pdf

Case Study #1: Beta Thalassemia - Survey Available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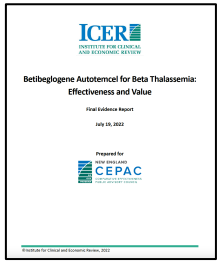

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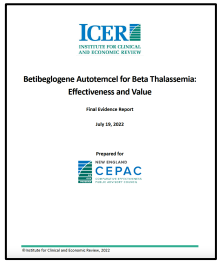
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Clickable link to the report…

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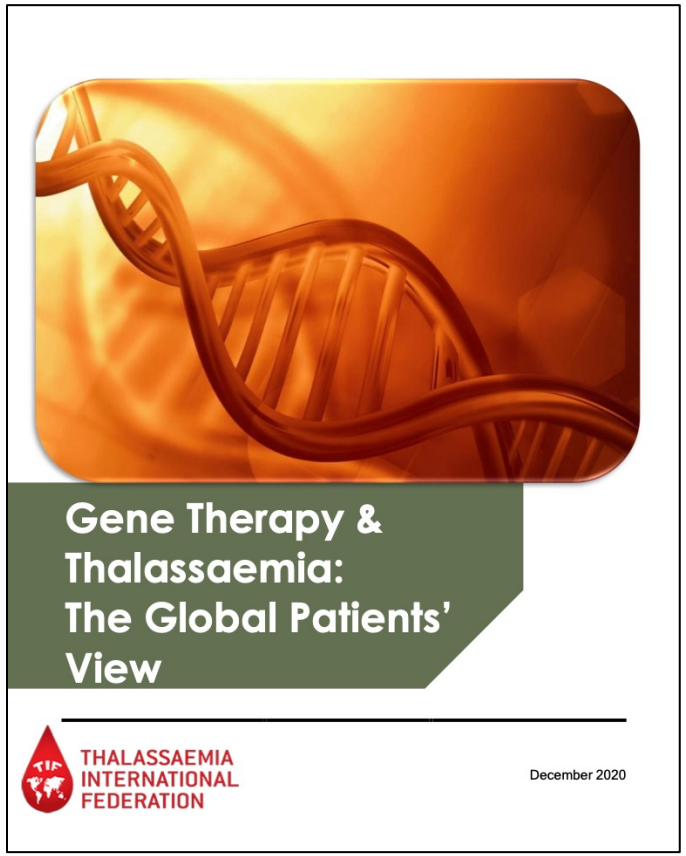

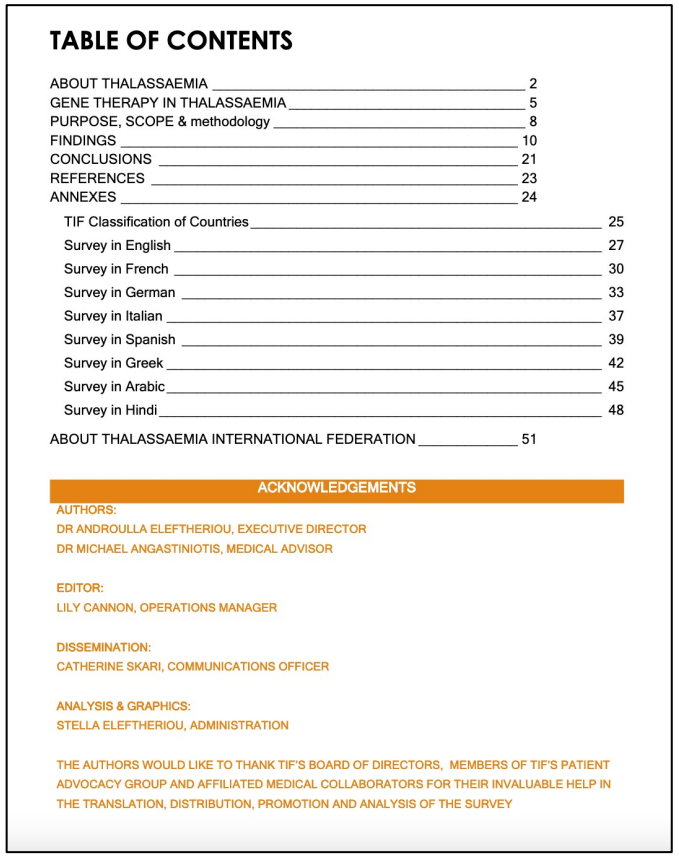

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ANALYSIS & GRAPHICS:
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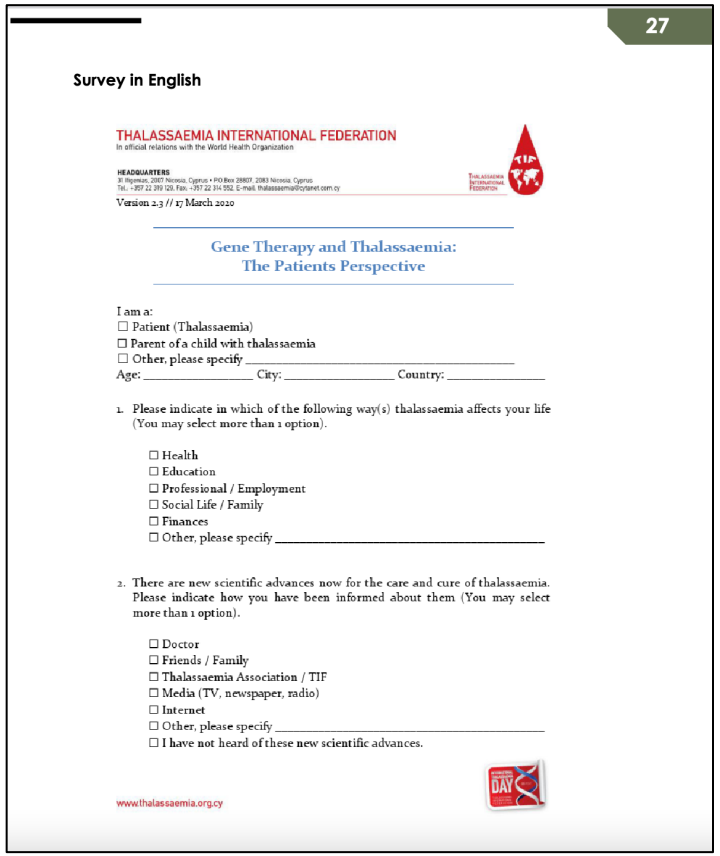
THE AUTHORS WOULD LIKE TO THANK TIF'S BOARD OF DIRECTORS, MEMBERS OF TIF'S PATIENT ADVOCACY GROUP AND AFFILIATED MEDICAL COLLABORATORS FOR THEIR INVALUABLE HELP IN THE TRANSLATION, DISTRIBUTION, PROMOTION AND ANALYSIS OF THE SURVEY



Survey in English

THALASSAEMIA INTERNATIONAL FEDERATION
In official relations with the World Health Organization

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Tel.: +357 22 319 123, Fax: +357 22 314 552, E-mail: thalassaemia@cytanet.com.cy

Version 2.3 // 17 March 2020

Gene Therapy and Thalassaemia:
The Patients Perspective

I am a:
☐ Patient (Thalassaemia)
☐ Parent of a child with thalassaemia
☐ Other, please specify _____
Age: _____ City: _____ Country: _____

1. Please indicate in which of the following way(s) thalassaemia affects your life (You may select more than 1 option).

☐ Health
☐ Education
☐ Professional / Employment
☐ Social Life / Family
☐ Finances
☐ Other, please specify _____

2. There are new scientific advances now for the care and cure of thalassaemia. Please indicate how you have been informed about them (You may select more than 1 option).

☐ Doctor
☐ Friends / Family
☐ Thalassaemia Association / TIF
☐ Media (TV, newspaper, radio)
☐ Internet
☐ Other, please specify _____
☐ I have not heard of these new scientific advances.

Source: https://thalassaemia.org.cy/wp-content/uploads/2020/06/GTSurveyReport_210212_FINAL.pdf

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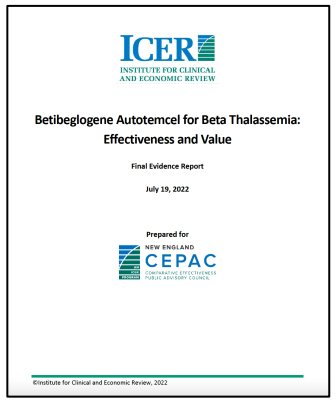
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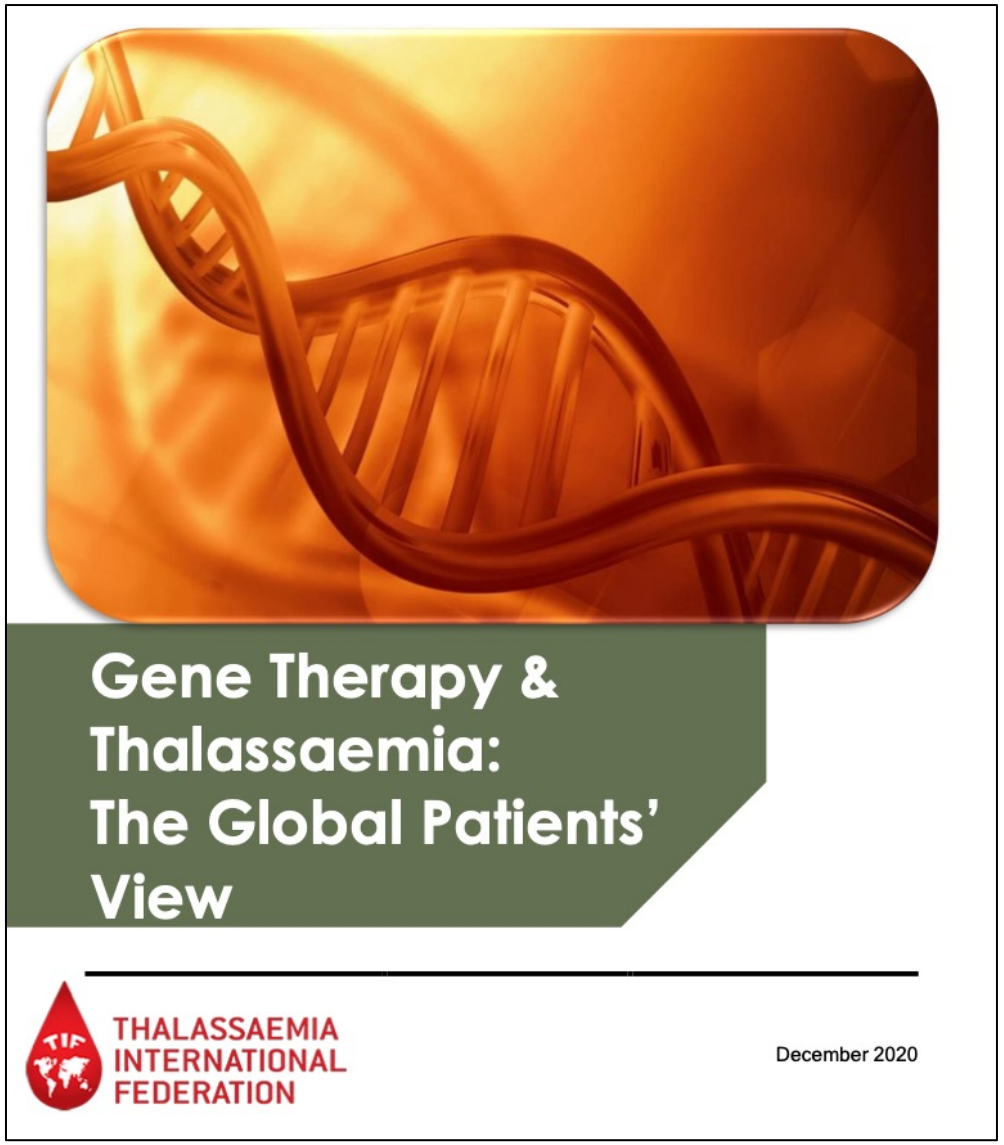

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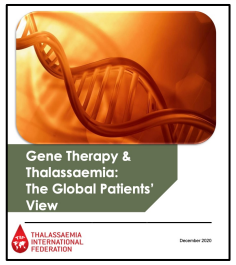

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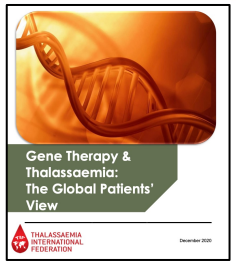

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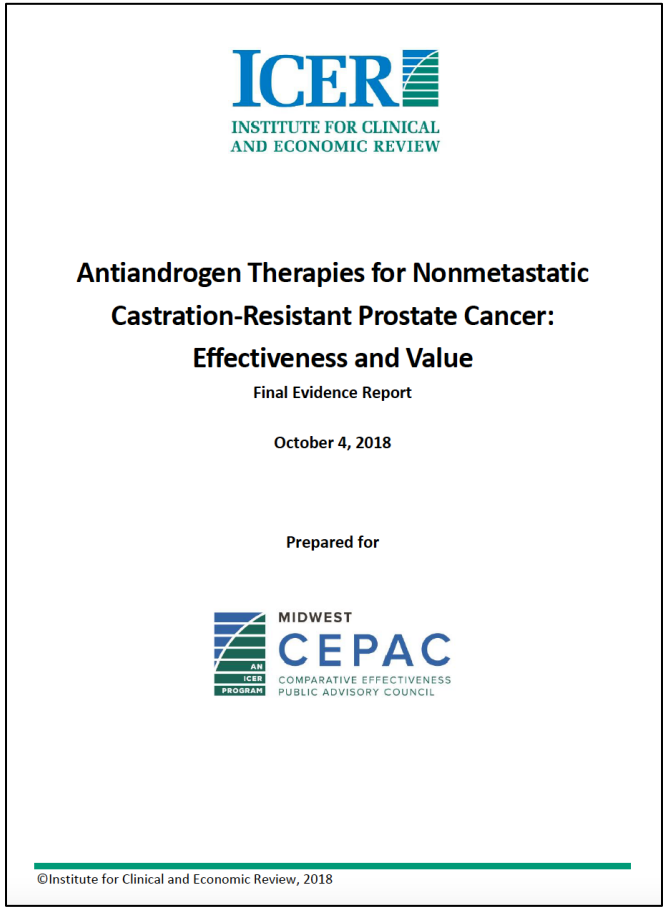
   ☐ Finances
   ☐ Other, please specify _____

Case Study #2: Prostate Cancer

Survey Not Available



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Antiandrogen Therapies for Nonmetastatic Castration-Resistant Prostate Cancer: Effectiveness and Value

Final Evidence Report

October 4, 2018

Prepared for

MIDWEST CEPAC
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL
AN ICER PROGRAM

Source: https://icer.org/wp-content/uploads/2020/10/ICER_Prostate_Cancer_Final_Evidence_Report_100418.pdf

Case Study #2: Prostate Cancer - Survey Not Available

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1.4 Insights Gained from Discussions with Patients and Patient Groups

Patients and patient groups stressed the serious risks of morbidity and mortality in men with CRPC and how this affects men and their families, the psychological effects of prostate cancer on a man's sense of self, the substantial side effects of therapies for prostate cancer, and the sense that burdensome therapy has failed when PSA levels begin to rise leading some to question their prior decisions about therapy.

In a 2017 survey report from the Cancer Support Community, 49 patients rated the following factors to be among the "most important" considerations when selecting a treatment for their prostate cancer: higher chance for survival (27%), higher chance for cure (20%), recommendations from a doctor (16%), and fewer side effects (14%).[17]

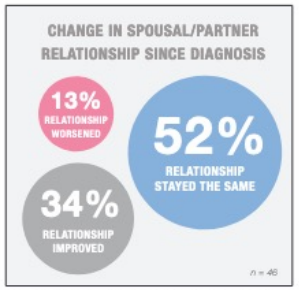
Prostate Cancer Specialty Registry Report 2017

What is the Prostate Cancer Specialty Registry?

Nearly 3 million men diagnosed with prostate cancer are alive today (American Cancer Society, 2017). The Prostate Cancer Specialty Registry, which began accepting participants in August 2015, documents the experiences of a cross-section of people living with prostate cancer. The Prostate Cancer Advisory Council—made up of prostate cancer specialists and other oncologists, behavioral scientists, patient advocates, and industry representatives—supports the efforts of the Registry by providing continued support and guidance on outreach, research, and the dissemination of findings (see a list of Advisory Council members at www.cancersupportcommunity.org/RegistryIndexReport2017).

CANCER SUPPORT COMMUNITY
A Global Network of Education and Hope

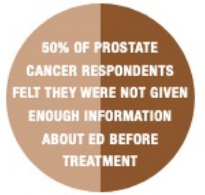
the wellness community



Erectile dysfunction (ED) is the most widely-known complication of prostate cancer—and, in fact, 73% of survey respondents reported an incidence of ED since their diagnosis. They were, by and large, optimistic about an effective treatment: 65% believed it was somewhat, quite a bit, or very much possible to treat ED. At the same time, 50% felt they were not sufficiently knowledgeable about ED prior to treatment.

SPOUSAL/PARTNER RELATIONS

Cancer-related sexual intimacy issues can lead to worsening communication, elevated emotional tension, and increased marital distress. Indeed, 21% of respondents reported being not at all comfortable sharing problems about ED with their spouse or partners, and 53% believed that ED has adversely impacted their relationship at least a little bit.

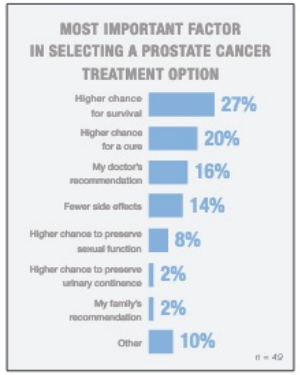


Further, an overwhelming majority (86%) reported that, in general, their cancer had put at least somewhat of an emotional strain on their spouse or significant other.

At the same time, many respondents indicated they make decisions about treatment with input and involvement from their spouse or partner: 44% made treatment decisions together, while 48% made decisions after consulting their spouse or partner.

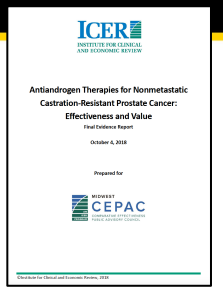
TREATMENT DECISION-MAKING AND PLANNING

There are a variety of options when it comes to prostate cancer treatment, including "watch and wait," surgery, radiation therapy, vaccines, hormone therapy, chemotherapy, cryosurgery, and bone-directed treatment. Twenty-two percent of respondents reported that they were under active surveillance ("watch and wait"). An even larger percentage (36%) were post-treatment, while 5% had recently been diagnosed and were currently making a treatment decision. Apart from active surveillance, 31% reported a current treatment of hormone therapy such as androgen deprivation



Sources: https://icer.org/wp-content/uploads/2020/10/ICER_Prostate_Cancer_Final_Evidence_Report_100418.pdf
https://www.cancersupportcommunity.org/sites/default/files/d7/document/the_prostate_specialty_report_7-10.pdf

Case Study #2: Prostate Cancer - Survey Not Available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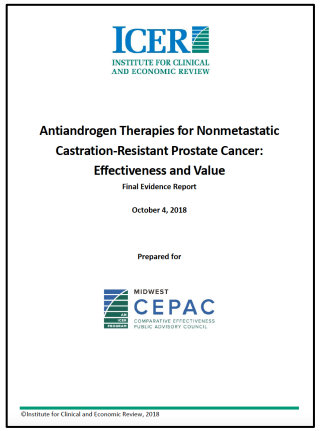
1.4 Insights Gained from Discussions with Patients and Patient Groups

Patients and patient groups stressed the serious risks of morbidity and mortality in men with CRPC and how this affects men and their families, the psychological effects of prostate cancer on a man's sense of self, the substantial side effects of therapies for prostate cancer, and the sense that burdensome therapy has failed when PSA levels begin to rise leading some to question their prior decisions about therapy.

In a 2017 survey report from the Cancer Support Community, 49 patients rated the following factors to be among the "most important" considerations when selecting a treatment for their prostate cancer: higher chance for survival (27%), higher chance for cure (20%), recommendations from a doctor (16%), and fewer side effects (14%).[17]

Referenced data source

Case Study #2: Prostate Cancer - Survey Not Available


ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

Antiandrogen Therapies for Nonmetastatic Castration-Resistant Prostate Cancer: Effectiveness and Value

Final Evidence Report

October 4, 2018

Prepared for

MIDWEST CEPAC
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL

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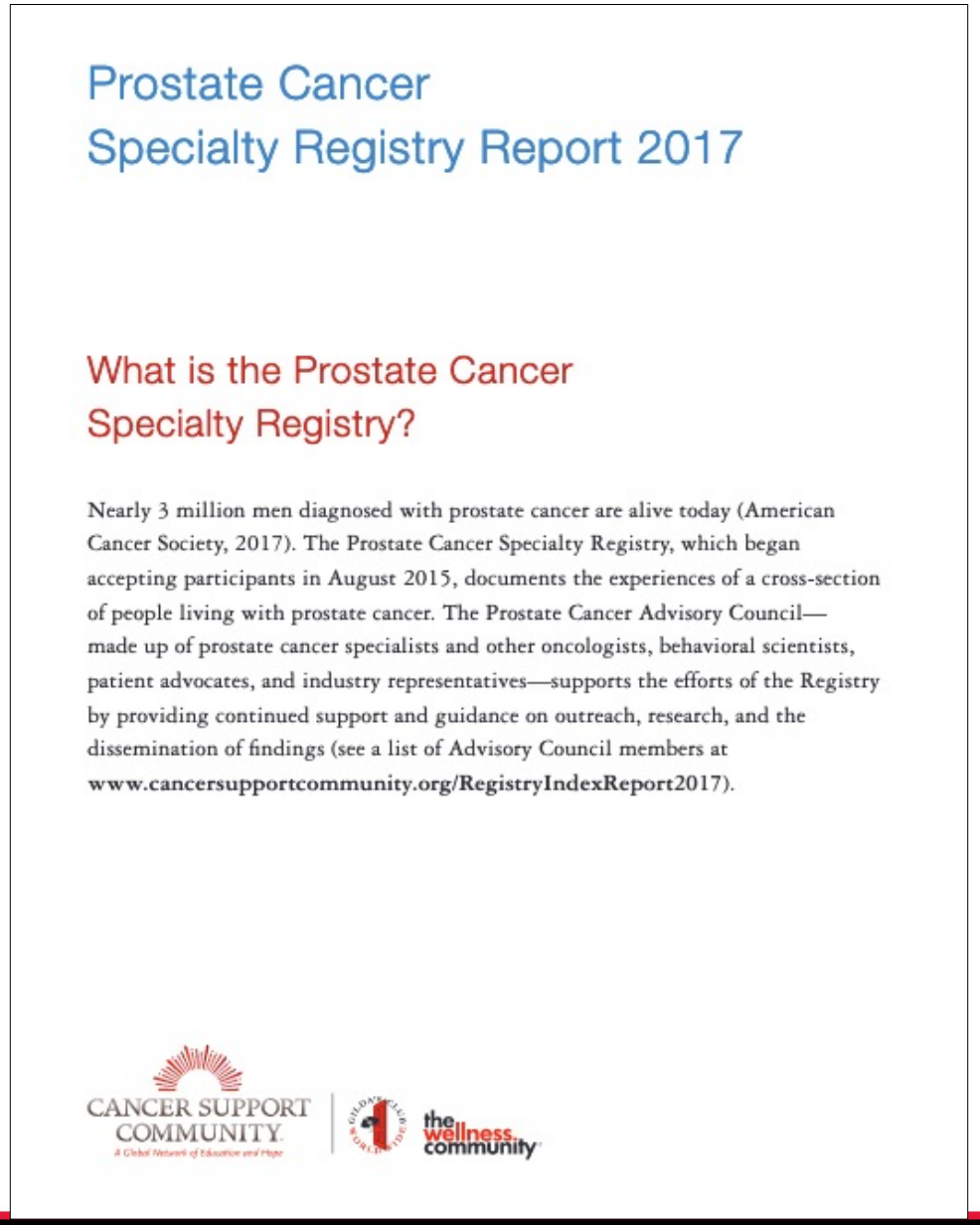
Referenced data source…


Prostate Cancer
Specialty Registry Report 2017

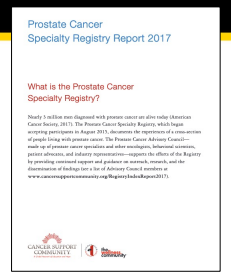
What is the Prostate Cancer Specialty Registry?

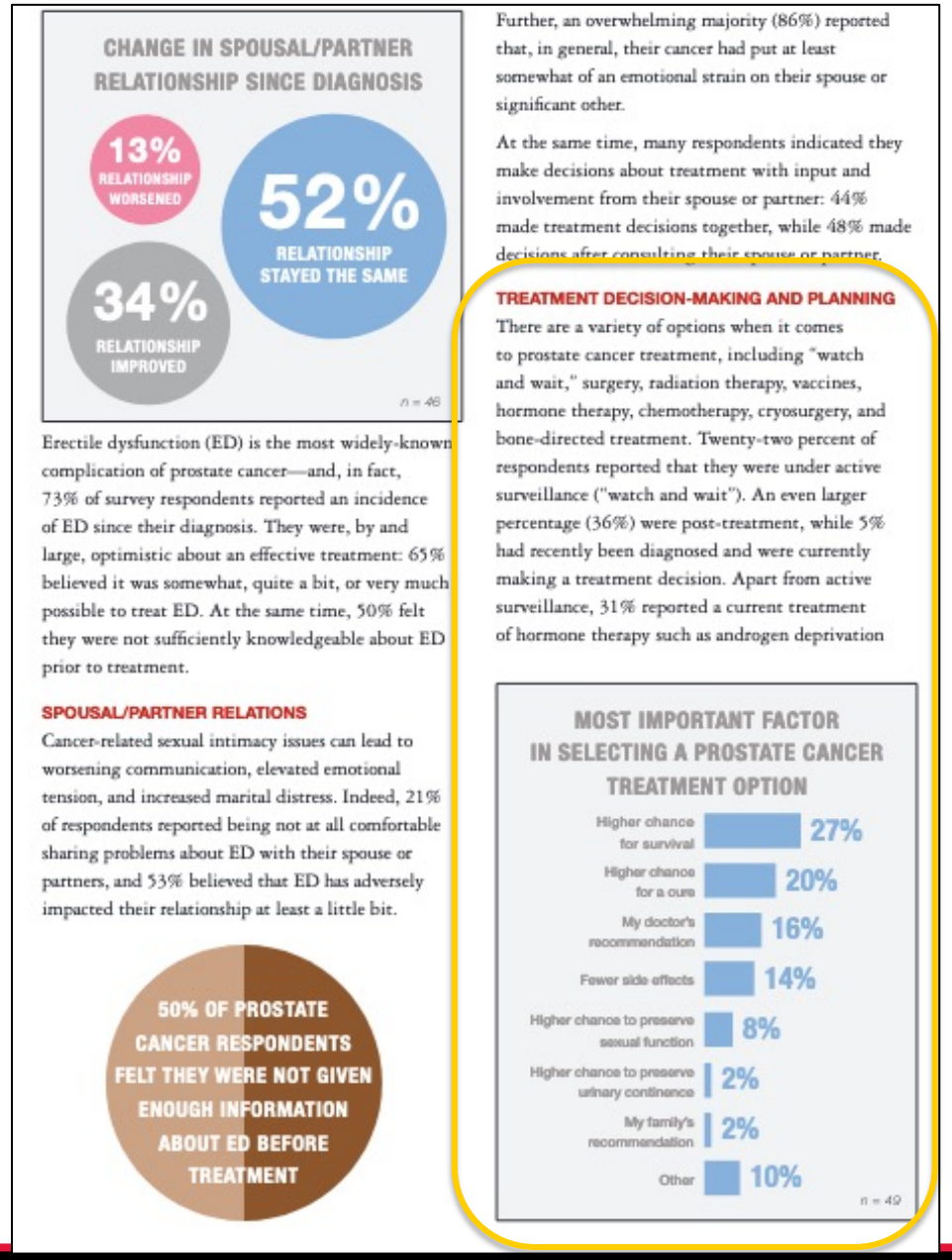
Nearly 3 million men diagnosed with prostate cancer are alive today (American Cancer Society, 2017). The Prostate Cancer Specialty Registry, which began accepting participants in August 2015, documents the experiences of a cross-section of people living with prostate cancer. The Prostate Cancer Advisory Council—made up of prostate cancer specialists and other oncologists, behavioral scientists, patient advocates, and industry representatives—supports the efforts of the Registry by providing continued support and guidance on outreach, research, and the dissemination of findings (see a list of Advisory Council members at www.cancersupportcommunity.org/RegistryIndexReport2017).

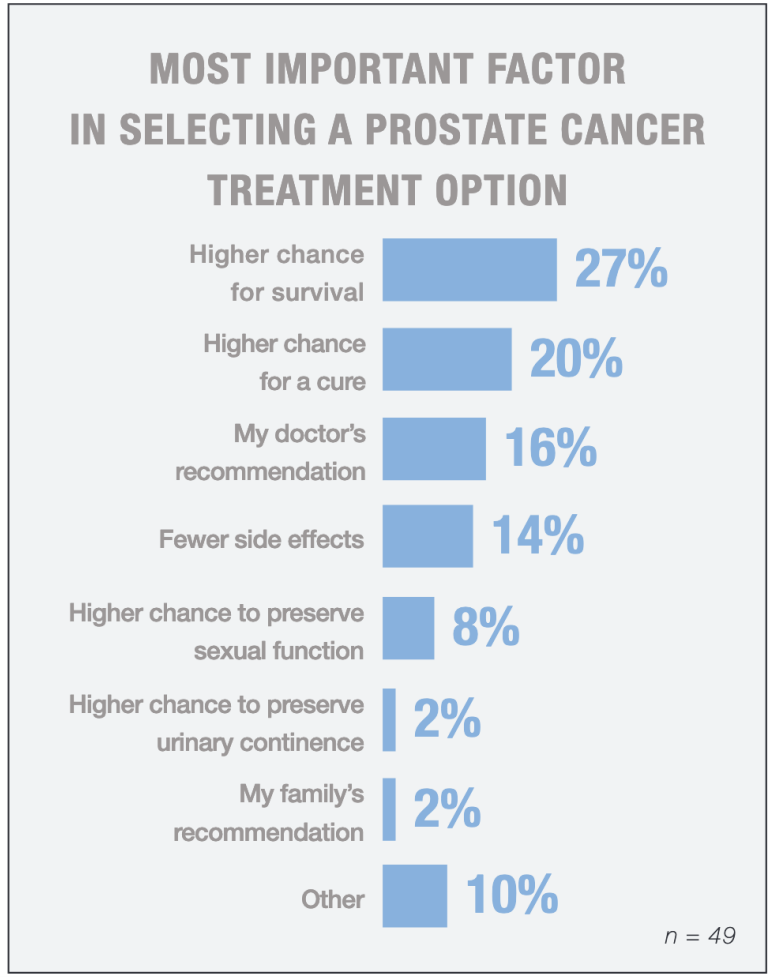
CANCER SUPPORT COMMUNITY
A Global Network of Education and Hope

the wellness community

Case Study #2: Prostate Cancer - Survey Not Available







Summarized information

Examples of Data Abstraction

2017 Final Evidence Reports

Descriptive Attributes for Abstraction:

- Condition
- Report date
- Report title
- Survey data from patient group
- Survey publicly available
- Report updated
- Condition revisited
- Reference

Institute for Clinical and Economic Review (ICER) Final Evidence Reports 2017 Assessments (n=8)							
Condition	**Report Date**	**Report Title**	**Survey Data***	**Survey Publicly Available**	**Report Updated**	**Condition Revisited**	**Ref**
Multiple Sclerosis: RRMS and PPMS	Mar 6, 2017	Disease-Modifying Therapies for Relapsing-Remitting and Primary-Progressive Multiple Sclerosis: Effectiveness and Value	Yes	Yes	-	2019 SPMS	1
Rheumatoid Arthritis	Apr 7, 2017	Targeted Immune Modulators for Rheumatoid Arthritis: Effectiveness & Value	Yes	No – summarized report	-	2019	2
Atopic Dermatitis	Jun 8, 2017	Dupilumab and Crisaborole for Atopic Dermatitis: Effectiveness and Value	No	-	-	2021	3
Osteoporosis	Jul 17, 2017	Anabolic Therapies for Osteoporosis in Postmenopausal Women: Effectiveness and Value	Yes	No – summarized report	-	-	4
Opioid Epidemic: Abuse Deterrent Opioids	Aug 8, 2017	Abuse-Deterrent Formulations of Opioids: Effectiveness and Value	No	-	-	2018, 2020, 2021	5
Ovarian Cancer	Sep 28, 2017	Poly ADP-Ribose Polymerase (PARP) Inhibitors for Ovarian Cancer: Effectiveness and Value	No	-	-	-	6
Chronic Pain: Lower Back & Neck	Nov 6, 2017	Cognitive and Mind-Body Therapies for Chronic Low Back and Neck Pain: Effectiveness and Value	No	-	-	-	7
Tardive Dyskinesia	Dec 22, 2017	Vesicular Monoamine Transporter 2 Inhibitors for Tardive Dyskinesia: Effectiveness and Value	Yes	Yes – select items	-	-	8

*Survey data from patient group

Results: Use of Patient-Experience Surveys in Institute for Clinical and Economic Review (ICER) Final Evidence Reports

		Survey use		*Survey availability*	
Year	Total reports	Reports with survey	%	Full	Partial
2017	8	4	50.0%	1	1
2018	12	4	33.3%	-	2
2019	9	2	22.2%	1	1
2020	11	1	9.1%	1	-
2021	10	3	30.0%	-	1
2022	6	2	33.3%	1	-
Total	**56**	**16**	**28.6%**	**4**	**5**

Use of patient-experience survey data did not increase across ICER value assessment reports from 2017-2022

Survey instruments were not always publicly available

Summary of Findings

- 16 of the 56 reports (28.6%) referenced patient-input data via patient surveys
- Frequency of survey use did not increase over time
  - In 2017, 4 of 8 reports used surveys compared to 2 of 6 reports used surveys in 2022
- For reports referencing survey data, public availability of instruments varied
  - 4 of the 16 (25.0%) survey instruments were publicly accessible
  - 5 of the 16 (31.3%) survey instruments were partially available beyond survey result summaries

Conclusions

- Use of patient-experience survey data appears to remain flat across ICER final evidence reports from 2017-2022
- Possible reasons for why survey use did not increase
  - Patient groups did not have capacity to collect data (e.g., during Covid)
  - Interviews or focus groups
- There can be a variety of reasons why instruments are not publicly available
  - Patient groups did not have resources
  - Third-party vendors involved
  - Proprietary instruments
  - Lack of awareness

Increased transparency and accessibility of patient-experience surveys can enhance understanding of which/how data have been collected and gaps in available data

Next Steps

- Discussions with patient groups involved in data collection to understand challenges or barriers to making instruments publicly available

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Thank you!

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