

Metastatic PPGL Survey

Feb 2024



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Advocacy Committee

- ▶ Formed in August 2023
- ▶ Response to the announcement that Lantheus has discontinued production of AZEDRA, the only FDA approved treatment for metastatic and unresectable pheo & para.
- ▶ Strategy(ies) may include advocating for:
 - ▶ better access to alternative treatments for metastatic patients such as:
 - ▶ PRRT
 - ▶ Conventional MIBG
 - ▶ Clinical trials
 - ▶ Advocate for the continued manufacture of Azedra
 - ▶ Patient education and support



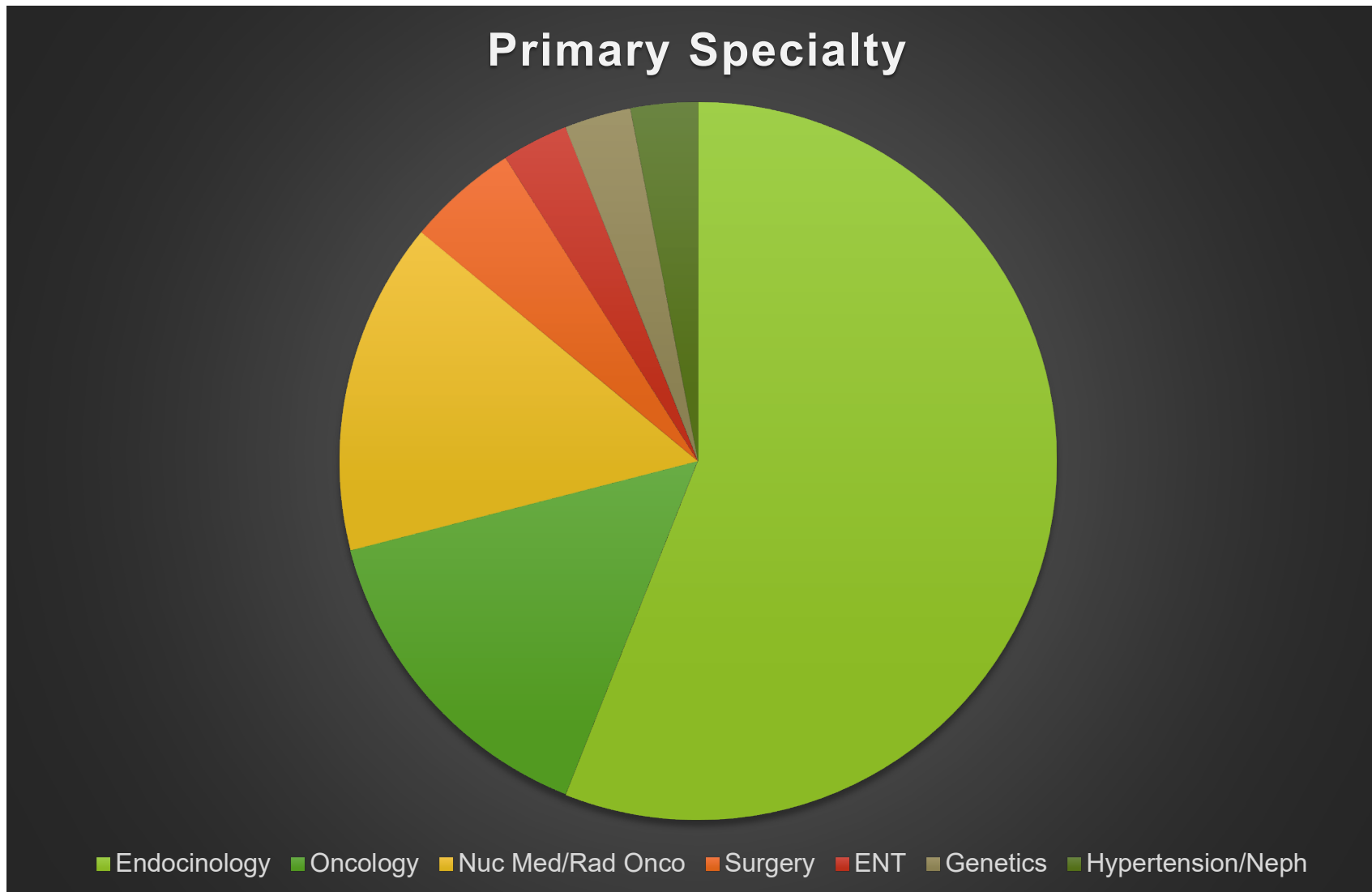
Metastatic PPGL Survey

- ▶ What: A survey of centers that treat metastatic PPGL patients
- ▶ Why: Gather information to develop a strategy to best support metastatic patients following the halt of production of Azedra
- ▶ Who: Centers worldwide that treat metastatic patients
- ▶ How: Survey Monkey survey shared via mass email to PPA healthcare provider email list & direct outreach to experienced PPGL centers including PPA [Centers of Excellence](#)
- ▶ When: Survey opened on 11/6/23 and closed on 1/16/24
- ▶ Responses shared are by respondent, some centers had multiple respondents per center

About the Respondents

Clinicians & Centers

53 respondents*



*some centers had multiple respondents

Respondents from 28 centers*

United States

- ▶ MD Anderson, Houston
- ▶ U. of Iowa
- ▶ Oregon Health & Sciences U. (2)
- ▶ University of Rochester
- ▶ University of Colorado (2)
- ▶ University of Utah (2)
- ▶ U. of Texas Southwestern
- ▶ NCI
- ▶ Stanford
- ▶ Penn (2)
- ▶ UCLA
- ▶ Dana Farber Cancer Institute (2)
- ▶ U. of Texas San Antonio (2)
- ▶ Mayo Clinic, Rochester
- ▶ Cleveland Clinic
- ▶ University of Florida

*responding with center name was not required

Europe

- ▶ Vall d'Hebron Univ. Barcelona, Spain
- ▶ St. Barts, UK
- ▶ Erasmus, MC, The Netherlands
- ▶ La Fe Hospital, Valencia, Spain
- ▶ University Hospital Wurzburg, Germany
- ▶ University Hospital Zurich
- ▶ Hopital Europeen Georges Pompidou, Paris
- ▶ General Hospital of Athens, Greece
- ▶ Univ. Hospital Dresden, Germany

Canada

- ▶ University of Calgary
- ▶ University of Toronto, Sick Kids Hospital

Asia

- ▶ Endocrine Institute Hospital Putrajaya, Malaysia

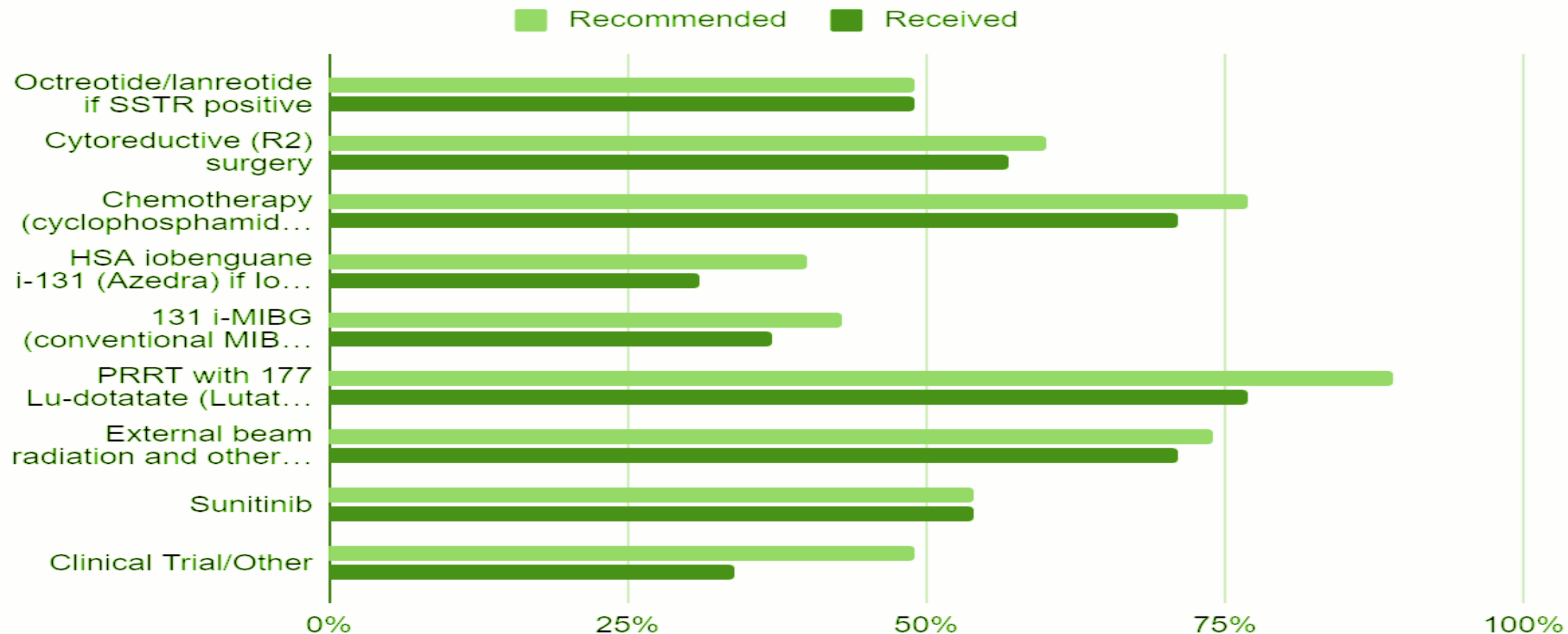


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Metastatic Treatment Options

Which of the following have you recommended vs. received for treatment of metastatic PPGL?*

*as recommended by NCCN guidelines



Is there anything else you would like us to know about the treatment of metastatic pheochromocytoma paraganglioma patients?

For example, you can provide additional detail about why a patient was recommended a course of treatment, but didn't receive it.

- ▶ *Difficult in UK to get PRRT and even sunitinib has to be sought under compassionate treatment programme.*
- ▶ *Insurance denial*
- ▶ *Lack of Azedra will be an issue*
- ▶ *Access to medical genetics and counselling for relevant patients with identified mutations*
- ▶ *Waiting list for PRRT due to limited funding*

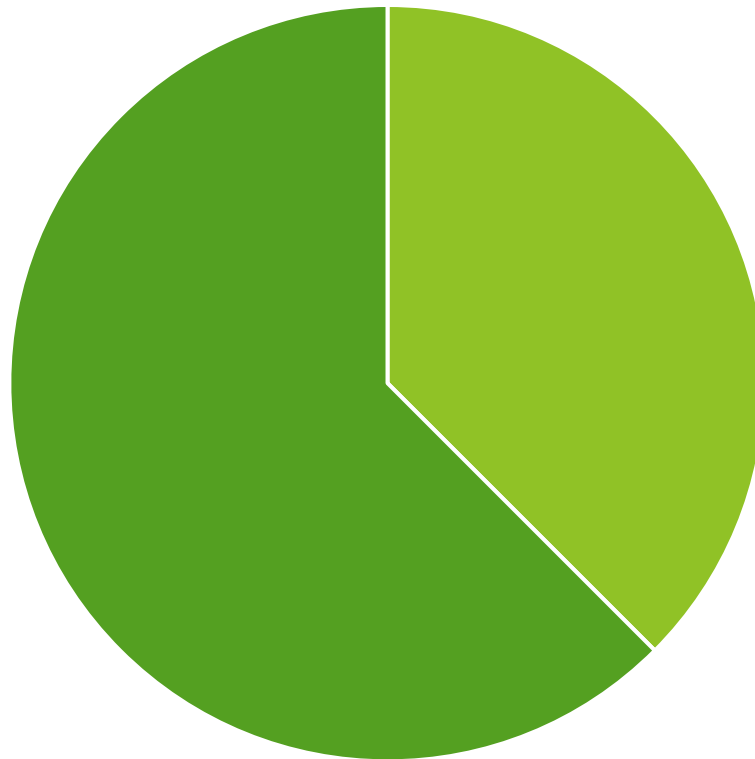
Is there anything else you would like us to know..., continued

- ▶ *Participation in clinical trials is encouraged; however, some health insurances do not cover participation trials or they do as long as the trial is a state where the insurance works (example Kaiser permanente covers trials in California but do not cover trials in other states). Lutathera is very expensive and Medicaid will not cover it because of its cost and Medicare may not cover it as is not FDA approved.*
- ▶ *We have also tried immunotherapy in patients (here insurance companies in Germany are more difficult)*
- ▶ *In Switzerland, the insurance usually does not pay for PRRT and MIBG therapy*
- ▶ *Limited therapeutic options for pediatric patients with metastatic PPGL as all are off-label!*

The background features abstract, overlapping green geometric shapes, primarily triangles and polygons, in various shades of green, creating a modern and dynamic visual effect. The shapes are layered, with some appearing more prominent than others, and they extend towards the corners of the frame.

About Azedra

Azedra Sites



9 Azedra sites responded
(out of ~16 Azedra sites)

■ Yes

■ No

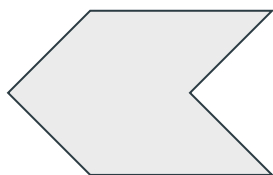


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Since FDA approval, how many patients did you treat with HSA iobenguane i-131 (Azedra)?

Approximation is ok. If none, please explain why you didn't treat patients with it.

- ▶ *none, patient had rapid disease progression requiring chemo, unstable for Azedra*
- ▶ *0 - it was set up to be given...before announcement, this was only for treating those up to age 25.15+*
- ▶ 5
- ▶ 7
- ▶ 8
- ▶ 30
- ▶ 44



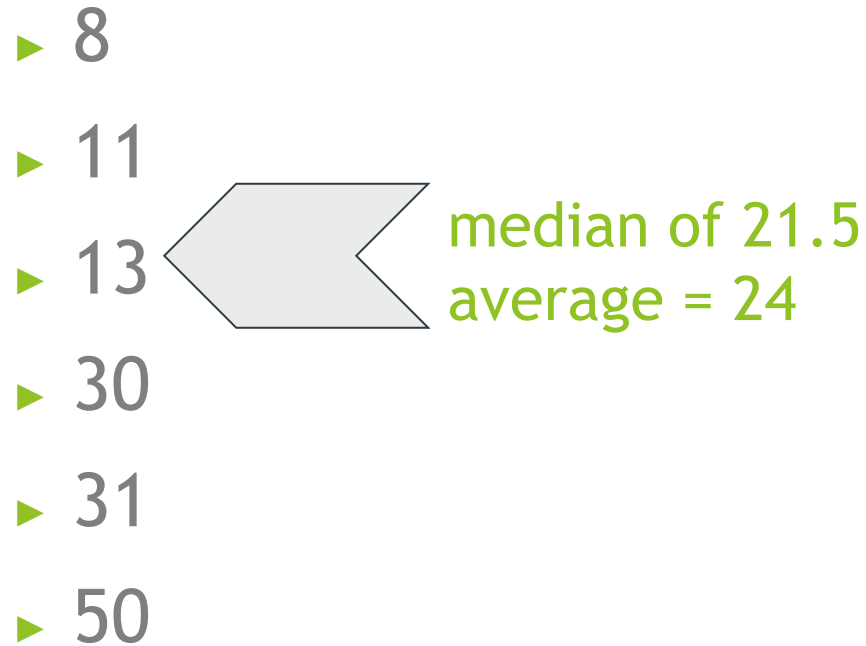
Of those that treated with Azedra:
median = 8 & average = 19



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Since FDA approval how many doses of HSA iobenguane i-131 (Azedra) did you administer?

Approximation is ok.



Is there anything else you would like us to know about your experience with HSA iobenguane i-131 (Azedra)?

- ▶ *Was discontinued before the patient was ready for azedra. we need it available*
- ▶ *Well tolerated*
- ▶ *Most patients where I used MIBG had poor somatostatin receptor expression and were not candidates for lutathera*
- ▶ *For adults, was difficult to get insurance to cover patients going out of state to get treatment when we did not have it in our state.*
- ▶ *The treatment can be complex/involved because of the dosimetry and need for hospitalization. But it has been well tolerated overall and effective for many of our patients.*

Is there anything else you would like us to know about your experience with HSA iobenguane i-131 (Azedra)?, continued

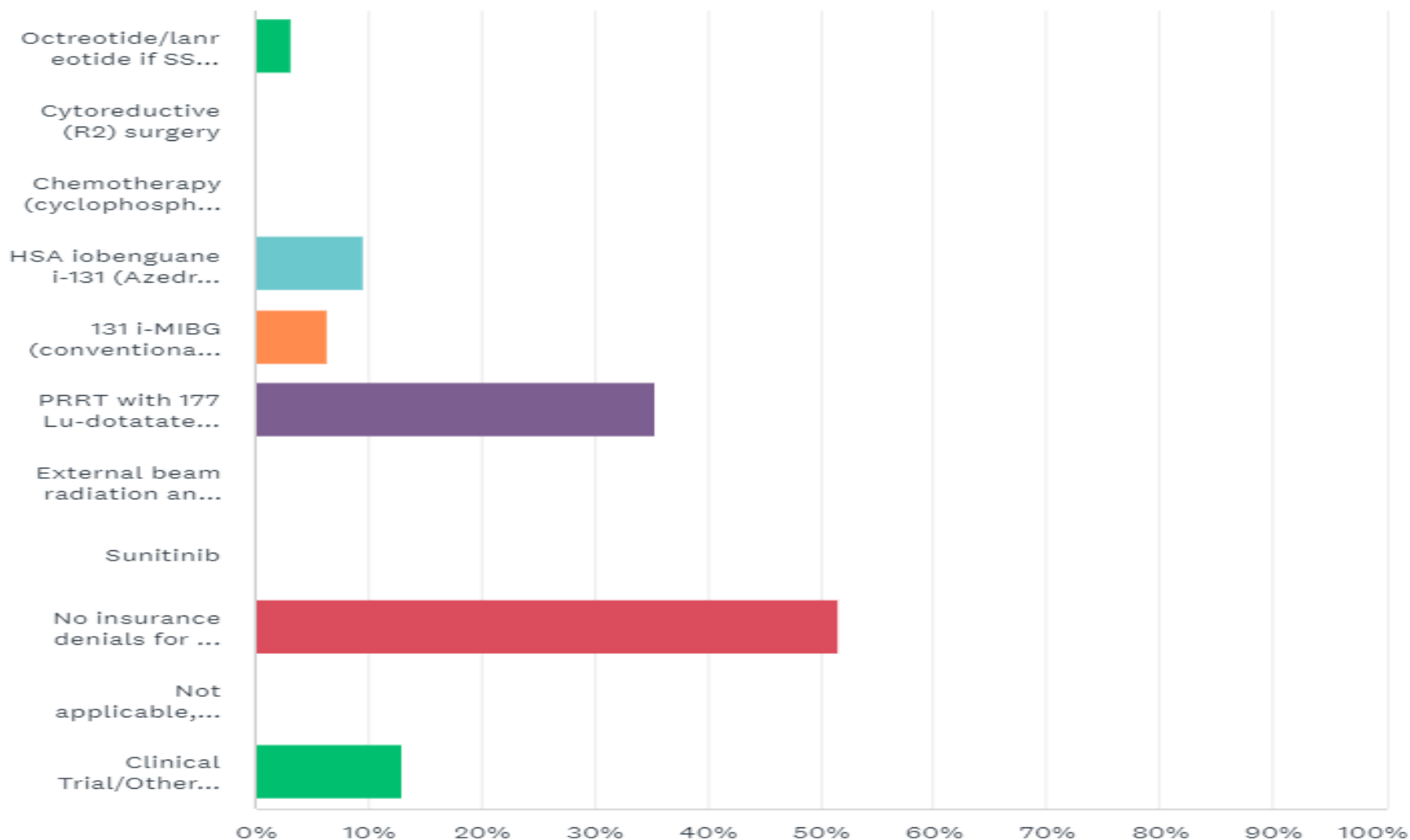
- ▶ *It is an effective medication. Most patients respond to therapy and responses can be durable. I have 5 patients with responses lasting 5 years; one patient has responded for 8 years and continues. Toxicity from a clinical perspective is acceptable as patients recover from bone marrow insufficiency. It is however, unclear how the long term toxicity may express (bone marrow dysplasia, leukemias. etc.). In my opinion is better than Lutathera. However, it will not be available any longer which is sad.*
- ▶ *It's an invaluable option for patients and we are very stressed at what to do when it is unavailable.*



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Insurance Coverage

Which of the following treatments have you recommended & received insurance denial for metastatic PPGL?



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Key Findings

Respondents

- ▶ Good representation of experienced centers
- ▶ Good representation from US (61%) & European Centers (30%)
- ▶ 56% of respondents were endocrinologists
 - ▶ Nuc Med/Rad Onc (15%)
 - ▶ Oncology (15%)

Metastatic Treatment Options

▶ Treatments recommended & received:

▶ Clinical Trials

▶ 49% recommended

▶ 34% received



15% difference

▶ PRRT

▶ 89% recommended

▶ 77% received



12% difference

Insurance Coverage

- ▶ 36% of respondents received insurance denial for PRRT
- ▶ 13% of respondents received insurance denial for clinical trial

Use of Azedra

9 Azedra sites responded

- ▶ Centers who treated with Azedra
 - ▶ Avg of 19 patients per center
 - ▶ Avg of 24 doses per center

Reasons for not providing/recommending Azedra to patients

- ▶ rapid disease progression, too unstable
- ▶ site setup right before discontinuation
- ▶ discontinued before the patient was ready
- ▶ insurance denial for out of state treatment
- ▶ complex/involved because of the dosimetry & hospitalization

Next Steps

- ▶ Share survey results with:
 - ▶ Respondents
 - ▶ Relevant industry partners
 - ▶ Medical Advisory Board
 - ▶ Office of Drug Shortage/FDA
- ▶ Identify barriers to access
- ▶ Share stories during Rare Disease Week
- ▶ Provide education resources focusing on:
 - ▶ access to PRRT & clinical trials
 - ▶ insurance pitfalls
 - ▶ external beam radiation

Thank You

Pheo Para Alliance is grateful to the clinicians who responded!

