# Sidney Kimmel Cancer Center Jefferson Health<sub>®</sub> | NCI – designated

### Background

Metastatic uveal melanoma (MUM) is a rare malignancy that has limited treatment option

Investigational (unapproved) medical products (IMPs) may offer a higher chance of benefit

We hypothesized that patients with MUM face challenges in accessing IMPs through clinic trial access (expanded access (EA)/compassionate use or right to try).

### Methods

We conducted a retrospective comparative electronic chart review for all living patients w and older, established at a single referral cancer center to assess MUM treatments includi trials.

We grouped patients by generation:

- Millennials: Age 18 to 41 years
- Generation X: Age 42 to 57 years
- Boomers: Age 58 to 76 years
- Silent Generation: Age 77 years and over .

The treatments were categorized:

- Liver-directed therapy with
  - Immunoembolization, with GM-CSF +/- IL-2
- Transarterial chemoembolization (TACE) with Carmustine
- TACE with doxorubicin eluting beads
- Yttrium-90 radioembolization
- Hepatic arterial infusion
- Radiation therapy at the tumor metastasis
- Surgical resection of tumors
- Standard systemic treatment
- Ipilimumab (3mg/kg) + nivolumab (1 mg/kg)
- Nivolumab (1 mg/kg or 480 mg monthly) and pembrolizumab.
- Tebentafusp (Kimmtrak) for patients with HLA-A2 positive, FDA, 2022
- Opdualag (PD1 nivolumab+ LAG3 Abs relatlimab) FDA, 2022
- IMPs through Clinical trial or EA.

Each category of MUM treatment was assigned a score of 1.

A descriptive analysis was conducted to explore the use of IMPs by generation age, sex, th the initial diagnosis of UM and metastasis, and the duration of illness since metastasis.

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## Preapproval Medical Products in Patients with the Diagnosis of Metastatic Uveal

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	Results						
ns. Tit than risk.		Age ra 18-41 3 7/154	inge years (4.5%)	Age range 42-57 years 33/154 (21.4%)		Age range 58-76 years 95/154 (61.7%)	Age range ≥77 years 19/154 (12.3%)
cal trials (CTs) or non-							
	Use of IMP through CT	2/7 (28.6)		18/33 (54.5)		32/95 (33.7)	2/19 (10.5)
ith MUM, aged 18 years ing IMPs in and out of	Use of IMP through EA	0/7(0)	)	0/33 (0)		3/95 (3.2)	1/19 (5.3)
			Time to metastasis < 1 year 24/154 (15.6%)		Time to metastasis > 1 year - <5 years 78/154 (50.6%)		Time to metastasis ≥ 5 years 52/154 (33.8%)
	Use of IMP through CT		10/24 (41.7)		25/78 (32.1)		19/52 (36.6)
	Use of IMP through EA		1/24 (4.2)		2/78 (2.6)		1/52 (1.9)
			Time since metastasis < 1 year 36/154 (23.4%)		Time since metastasis > 1 year - < 5 years 94/154 (61%)		Time since metastasis ≥ 5 years 24/154 (15.6%)
e time elapsed between	Use of IMP throug	h CT	8/36 (22.2	2)	36/9	94 (38.3)	10/24 (41.7)
viously presented at the 2023	Use of IMP through EA		0/36 (0)		2/94 (2.1)		2/94 (2.1)

### Conclusion

In this center, a substantial number of patients with MUM received IMPs through CTs (35%) compared with the national U.S. average of only 6.3% of patients with cancer enrolled in cancer treatment trials from 2013 to 2017 (Unger & Fleury, 2021). However, the number of patients with MUM who received IMPs through EA was small (2.6%) and should be improved. EA should remain a viable option for patients who do not fit the trial requirements or do not have realistic access to a trial site. Thus, we need a better system to increase the number of patients who received IMPs through EA. More research is needed to see if our findings are generalizable to other patients at other institutions. References Congressional Research Service. (2021). Expanded access and Right to Try: Access to investigational drugs. https://sgp.fas.org/crs/misc/R45414.pdf National Library of Medicine (2023). ClinicalTrials.gov. https://beta.clinicaltrials.gov/search?distance=50&cond=Uveal%20Melanoma Nshimiyimana, R., C.E. Guzzetta, C.E., Brown, M., Zhou, Q., Johnson, J.M., Sato, T., Keith, S. (2018). Pilot study of anxiety, depression, and quality of life in patients with the diagnosis of metastatic uveal melanoma. Annals of Oncology (2018) 29 (suppl\_8): viii557-viii561. 10.1093/annonc/mdy296

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Access (CUPA)