



## **TOPIC: Press Release, New Report for Uveal Melanoma Patients**

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Media Contact:

Deanna Darlington, CEO

Deanna.Darlington@haystackproject.org

### **New Report Reveals Uveal Melanoma Patients Need Treatment, Genetic Testing and Additional Clinical Trials**

**Washington, DC January 3, 2022** — Haystack Project’s new Patient Oriented Value© Report was prepared for and in collaboration with the Melanoma Research Foundation and its CURE OM initiative. The report focuses on Uveal Melanoma, and reveals that although patients have access to providers and treatments for their primary tumor, more needs to be done to ensure that available tools to assess metastatic risk are covered by insurers, and expand access to clinical trials for adjuvant treatments and emerging therapies for metastatic disease.

Ocular melanoma (OM) is a rare, non-cutaneous melanoma that occurs in all races and ages. Uveal melanoma (UM) accounts for 95% of OM cases. Primary UM tumors can be treated successfully with radiation therapy or enucleation (removal of the eye). Unfortunately, UM patients have up to a 50% risk of metastasis following treatment of their primary tumors, and there is no curative treatment for metastatic UM. Given the high overall risk of metastasis, all UM patients enter into an uncertain “wait and see” surveillance period of periodic or routine monitoring for metastases, which most often occur in the liver. Genetic or molecular prognostic testing of the primary tumor (e.g., DecisionDX-UM), capable of stratifying patients into high, medium or low risk for metastatic disease, has been commercially available since 2009 and can be used to inform the frequency and intensity of surveillance protocols during the “wait and see” period.

“[C]overage for genetic or molecular prognostic testing may vary from payer to payer and require UM patients to go through multiple appeal processes to secure coverage. Medicare covers the DecisionDX-UM test but requires that billing providers utilize a registry and implies a level of oversight on subsequent care, including referrals and follow-up surveillance intensity, which could deter utilization.”

Knowledge of a patient’s risk of developing metastatic disease can also help patients and their clinicians determine whether participation in an adjuvant therapy clinical trial would be of potential benefit. As of this report, two adjuvant therapeutic regimens are under clinical development for UM patients at high risk for metastases.

“Although there is no current consensus on a standard of care for adjuvant treatment in high-risk patients, 14 of the 35 high-risk respondents (40%) indicated that they have received adjuvant treatment, either within or outside of a clinical study. This contrasts sharply with the reported experience of patients with “unknown” risk of metastatic disease. None of these patients have received adjuvant treatment or have enrolled in a clinical trial.”



Treatment protocols recommended by their doctor and that successfully eradicate primary tumors while sparing the eye and vision are extremely important to UM patients. Factors such as involvement of pain/discomfort, repeat therapy administration or clinic visits, bothersome side effects, and the requirement for travel were less important in determining the primary tumor treatment.

“[N]ewly diagnosed UM patients are not always informed that genetic testing for metastatic risk is available. In addition, a subset of respondents indicated that their metastatic risk was “unknown” despite reporting that genetic testing was performed. This finding suggests a gap in communication that could be addressed by augmenting clinician-led discussions on diagnosis, treatment planning and follow-up with resources that can be taken home and reviewed, such as the *Ocular Melanoma Patient Guide* developed by CURE OM (the guide can be downloaded from: [Diagnosis \(Ocular\) | Melanoma Research Foundation](#)).”

Changes in vision were not reported as having a significant impact on patients’ employment or ability to live independently; however, approximately one third of patients across all risk groups for metastatic disease reported that activities of daily living were reduced due to changes in vision. As predicted, 50% or more of those with, or at known high risk for, metastatic disease reported less ability to plan for the future because of the uncertainty of their disease progression. However, those with known low risk expressed more confidence in this area. The majority of respondents at various risk for metastatic disease, and especially those with metastatic disease, also reported occasional dependence on a spouse, partner, child and/or caregiver.

UM Patients live with the possibility of developing metastatic disease for years, and even decades, after their primary tumor is diagnosed and successfully treated. Advocacy organizations such as the Melanoma Research Foundation are an important resource for patients seeking information as they navigate their care and as a conduit between patients, clinicians, researchers, payers, and regulators.

“[T]he long timeline from diagnosis to emergence of metastatic disease suggests that clinical trial designs for potential adjuvant therapies could require durations to or beyond 5 years to demonstrate efficacy. This underscores the need for reliable surrogate biomarkers indicative of emerging metastatic disease for use in UM adjuvant clinical trials. Additionally, collaborative strategies between FDA, industry, researchers, clinicians, and patient advocacy organizations to expand early access to the full addressable population would offer hope to UM patients and may enable data collection outside the clinical trial context.”

“Although expanded access programs are designed to get treatments demonstrating safety and efficacy in clinical trials to the patients needing them, patient awareness of, and therefore access to, these programs often depend upon clinician expertise in the disease. Patient advocacy organizations can play a pivotal role in alerting patients to therapeutic advances and providing the key information patients need to navigate access.”



### ***About***

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate efforts that address systemic value and access barriers. Our core mission is to evolve health care payment and delivery systems to make innovation and quality treatments accessible to all Americans living with or caring for someone with a rare or ultra-rare condition. We strive to amplify the patient and caregiver voice in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic.

The Rare Cancer Policy Coalition (RCPC) is a Haystack Project initiative, and the only rare cancer coalition developed to focus on and respond to access and value issues across the rare cancer community. RCPC also gives participants a platform for focusing on emerging landscape changes that impact new product development for rare cancers. Working within the Haystack Project enables RCPC participants to leverage synergies and common goals with other rare and ultra-rare patient advocates.

POV<sup>®</sup> Reports are undertaken to provide insight into the patient journey, articulate disease burden from the patient perspective, reveal real-world care gaps and communication deficiencies, and better understand treatment priorities and perceived value from the patient perspective.

Founded in 2011, CURE OM (the Community United for Research and Education of Ocular Melanoma) is the Melanoma Research Foundation's initiative to increase awareness, education, and research funding for ocular melanoma, while improving the lives of people affected by this disease.

The Melanoma Research Foundation (MRF) is a 501(c)(3) non-profit organization advocacy organization. Its mission is to eradicate melanoma by accelerating melanoma research while educating and advocating for the melanoma community. The foundation's goal is to transform melanoma from one of the deadliest cancers to one of the most treatable.

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