CLEAR CELL SARCOMA: PATIENT REPORTED REAL WORLD EVIDENCE

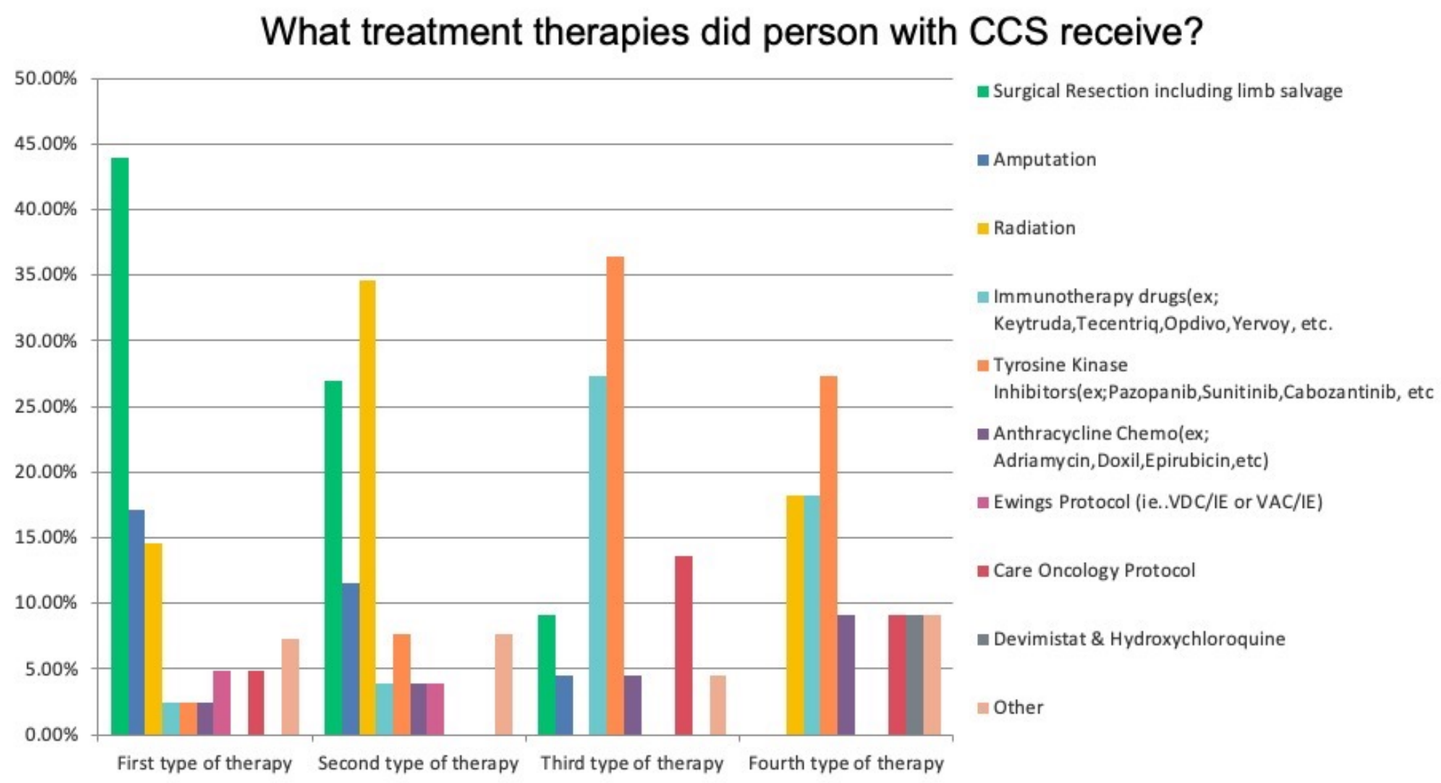
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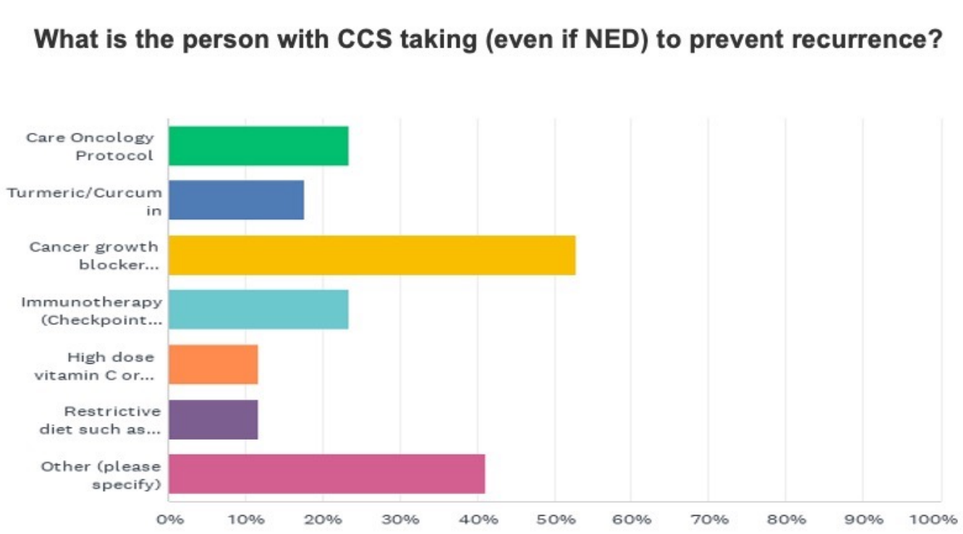
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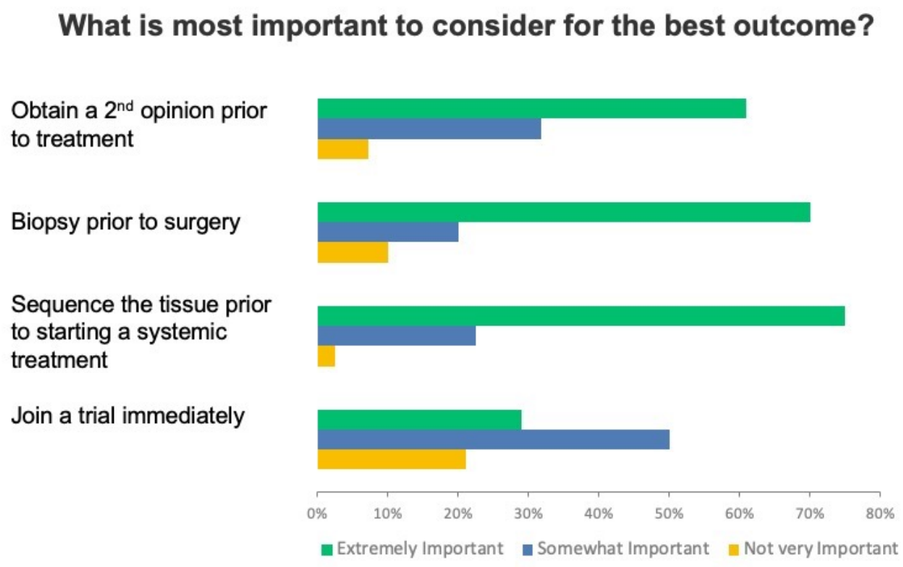
Objective: Clear Cell Sarcoma (CCS) is an ultra-rare and aggressive soft tissue sarcoma which typically develops in tendons or aponeuroses, and affects mostly teens and young adults. In a majority of cases, CCS is caused by a translocation resulting in the fusion of the genes for activating transcription factor 1 (ATF1) and Ewing sarcoma breakpoint region 1 (EWSR1), resulting in the EWSR1-ATF1 fusion protein. Since CCS is so rare, there is a paucity of research, leading to few treatment options that are generally based on weak scientific evidence. The CCS patient community is eager to Share RWE data as reported by CCS patients and/or their primary caregiver with hopes to initiate further patient data collection and disease natural history research with clinicians, researchers and patients working together. Persons with Clear Cell Sarcoma have been sharing their lived experiences with each other for more than ten years on a private Facebook page and want the sarcoma community to have access to that Real World data through this presentation for the purpose of improving patient outcomes.

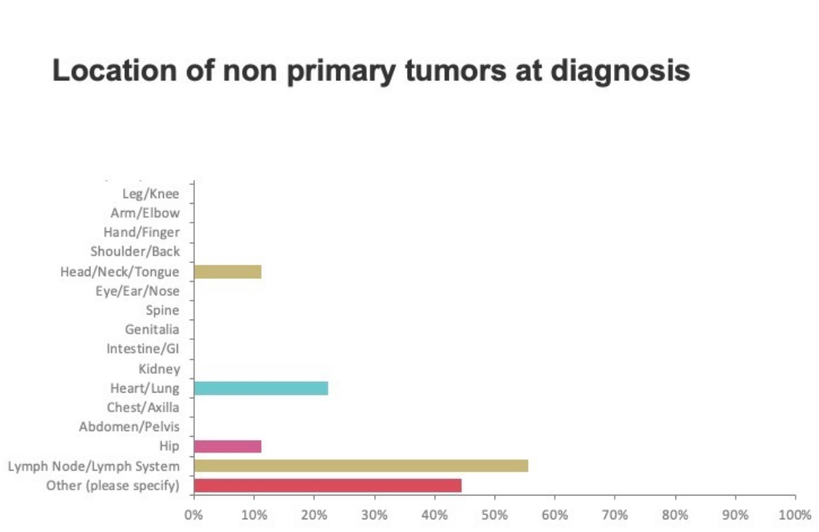
Methods: An anonymous electronic survey was created using a basic questionnaire originally designed in 2010 by the founding members of the CCS family Facebook page along with additional input from the Clear Cell Sarcoma Foundation (CCSF) Medical Advisory Board. Survey respondents include active participants in the CCS family private Facebook group (310 members) or have reached out to the CCSF through the CCSF website. The survey was administered via a HIPPA compliant Survey Monkey platform. CCS patients and caregivers were given a dedicated email address to request the survey for participation. All responses are de-identified through the platform.

Results: Sixty surveys were requested and 42 completed at the time of poster preparation. At the time of diagnosis, 83% of the respondents were under the age of 45, 44% were under the age of 25 and 32% under the age of 18. Sixty percent of the respondents were female. Eighty three percent of the female respondents with previous pregnancy had CCS symptoms during pregnancy or soon after. Fifty three percent of all respondents had disease localized to extremities, 24% found to have metastatic disease at diagnosis, 60% of patients had a visible lump prior to CCS being diagnosed and 60% had pain in the area of the tumor. Seventy one percent of respondents had a difficult time getting a diagnosis and said it took months/years to get a correct CCS diagnosis. Eighty four percent of misdiagnosed CCS patients were originally misdiagnosed with either Melanoma or Ewing Sarcoma. The correct diagnosis for CCS is often made after the standard of care treatment for the misdiagnosed disease is given. Twenty four percent of respondents found errors in their medical records. Ninty seven percent of CCS patients surveyed want to be involved in research and are willing to give their time and share information with researchers and clinicians who want to find better diagnostic tests and treatments for CCS.









Conclusion:

- Many research projects put a high value on gathering medical data directly from patients charts or the treating institutions in an effort to minimize patient reported inaccuracies. 24% of CCS patients report they are misdiagnosed or intentionally diagnosed with something other than CCS in a well-meaning effort to ensure insurance will allow the treatment prescribed by the treating physician. The CCSF recommends patients and researchers collaborate on projects where CCS patient data is gathered to ensure accurate information is being used. This includes retrospective data.

- Bloodwork is a relatively quick and easy way for the general health of CCS patients to be monitored and for researchers to develop diagnostic assays to monitor disease and treatment response.

- A majority of CCS patients had a lump and more had pain in the area of the tumor before diagnosis. Sarcomas are generally thought of as not producing pain which can lead to misdiagnosis as a sprain or injury, thus prolonging proper treatment.

- Twenty four percent of CCS patients are diagnosed with metastases and of those metastases 55% were in the lymph nodes and 22% in the lung

- Eight CCS patients (36%) of 22 respondents had some type of radiation treatment, of those, 3 (38%) had neoadjuvant radiation prior to surgery and are currently NED. Three (38%) had adjuvant radiation after surgical resection and ccs returned within one year. One patient reported radiation as palliative care and 1 patient had combination therapy with radiation prior to surgery and reported increased tumors not seen on previous imaging.

- 25% of respondents are taking the care oncology protocol to prevent CCS from returning

- Nearly all of the patients queried responded positively about being involved in medical research related to their CCS diagnosis. The CCSF encourages collaboration and open discussions with the CCS family of patients. Please reach out by sending an email to info@clearcellsarcoma.org to set up a focus group or discuss how we can help each other.

*Acknowledgments:
A special thank you to our CCSF Medical Advisory Board for their thoughtful advice and input and also to the CCS Family of patients and families dedicated to helping each other live better. We Dedicate this work to the CCS family that began this project in 2010, Caro D,Mike G,Bret H,Chris Z,Carol B,Dani S,Ashley A,Doug R,Kristen S,Darryl K,Danielle D,Rachel R.Wei. And Scott Heller of the CCSF.