

Rare Blood Cancers in 2021: Importance of Continued Exchange of Ideas

Naveen Pemmaraju

Department of Leukemia, The University of Texas MD Anderson Cancer Center, Houston, TX, USA

Address correspondence to Naveen Pemmaraju (npemmaraju@mdanderson.org).

Sources of support: None. Conflicts of interest: Consulting/honorarium from Pacylex Pharmaceuticals, Incyte, Novartis, LFB Biotechnologies, Stemline, Celgene, AbbVie, MustangBio, Roche Diagnostics, Blueprint Medicines, and DAVA Oncology; research funding/clinical trials support from Affymetrix, SagerStrong Foundation, Novartis, Stemline, Samus, AbbVie, Cellectis, Daiichi Sankyo, and Plexxikon.

Received: Jun 21, 2021; Accepted: Jul 1, 2021

Pemmaraju N. Rare blood cancers in 2021: importance of continued exchange of ideas. *J Immunother Precis Oncol.* 2021; 4:115–116. DOI: 10.36401/JIPO-21-X6.

© Innovative Healthcare Institute

It is my distinct pleasure to introduce this inaugural special issue of *JIPO* dedicated to the emerging and important issues in the field of malignant hematology including an emphasis on rare blood cancers. As we continue our journey together through the global pandemic of COVID-19, we have once again re-discovered the importance of scientific innovation, the necessity of rigorous exchange of ideas, and the vital need for ongoing discussions and debates in each of our academic and scientific sub-fields of interest. Novel methods of communication have been instrumental in moving the scientific processing of rapid-fire information, including online, social media, and even newer audio-based platforms, all aimed at bringing various stakeholders across the healthcare fields together virtually, even when in-person meetings were rendered unsafe or impossible. One method that clearly remains vital in our modern-day scientific discourse is the exchange of ideas via journals and journal articles; now accessible not just in one's library but available virtually anytime, anywhere—on one's laptop, one's desktop computer, even one's smartphone. The continual improvement of the accessibility of information, which of course was starting prior to the pandemic but was clearly accelerated by the pandemic, and the agile re-imagination and re-creation of conferences and meetings in hematology/oncology by our colleagues foraging into the completely virtual space, with all of its expected initial technical glitches and limitations, has truly allowed for a democratization of scientific information across geographic and other barriers. In this way, the urgent need for information and new ideas in rare blood cancers has been given new life in the digital information age, whereby experts can reach out directly to patients and caregivers, with the most rare of rare diseases, in hopes of finding each other.

One such example I would like to highlight in 2021 is one of my own expertise areas, a field in which I have

dedicated a decade-plus of focused work with my growing team, that of blastic plasmacytoid dendritic cell neoplasm, or BPDCN. Known as a rare blood cancer with historically poor outcomes, this clinically aggressive hematologic malignancy has experienced multiple name changes and difficulty in diagnostic specificity over the past three decades. Now taking it place as an officially recognized entity, as its own separate hematologic malignancy, the BPDCN field has experienced a tremendous amount of novel discovery and new approaches, with direct benefit to our patients and future directions in this emerging area. The discovery of universal expression of CD123 (IL3R alpha) as a surface marker/target on all BPDCN cells directly led to the investigation and establishment of the first targeted agent in this field (DT-IL3/SL401/Tagraxofusp) approved by US FDA in December 2018 for patients ages 2 and older with BPDCN. Further developments in the field have really taken off since that time, including many new CD123-targeted agents in development, identification of other targets beyond CD123 such as BCL-2 in BPDCN, and increased awareness and recognition in the rare disease field, all to the ultimate benefit for patients, caregivers, and others involved in this novel research area. Discoveries being made in the BPDCN and CD123 fields have opened the door to research discoveries in several other areas^[1]. Insights in rare diseases often help us understand and extrapolate ideas into the pathobiology and pathways of other more commonly occurring cancers, further highlighting the need for cross-pollination of ideas among the sub-fields and continued investment of resources into all areas of science, no matter how common or rare. This pandemic era has taught us once again that we must always follow the truth, we must follow the science, we must follow changes in our fields and be dynamic and nimble enough to recognize changing patterns and be prepared to make adjustments together.

In this special issue of *JIPO*, I am thrilled that we have included papers highlighting the fields of two emerging areas of rare blood cancer research in chronic myelomonocytic leukemia (CMML) with emphasis on hematopathologic collaboration and myeloproliferative neoplasms (MPNs) with emphasis on the rise of understanding of novel pathways, new thoughts on drug discovery, and novel clinical trials^[2-4]. Finally, I am very happy to feature a cutting-edge review of the practice-changing area of CAR-T and immunotherapy, with focus on real-world experience in this paradigm-shifting therapeutic area^[5,6]. I am thankful to Dr Aung Naing and team for allowing me to lead this special issue, and I am honored to introduce this dedicated review of rare blood cancers, because at the end of the day, it doesn't matter if a disease is "rare" or "common" it only matters if you or your loved one are facing the disease; and for you to know that there are researchers, doctors, healthcare providers and other stakeholders out there who are working day and night on it. It is time that we ensure the exchange of ideas and scientific discourse continues in the post-pandemic era and that we always strive to get information to each other and those who need it the most.

References

1. Pemmaraju N, Lane AA, Sweet KL, et al. Tagraxofusp in Blastic Plasmacytoid Dendritic-Cell Neoplasm. *N Engl J Med*. 2019;380:1628–1637.
2. Lee SS, Verstovsek S, Pemmaraju N. Novel therapies in Myeloproliferative Neoplasms: Beyond JAK Inhibitor Monotherapy. *J Immunother Prec Oncol*. Published online June 29, 2021. 2021;4:117–128.
3. Kuykendall AT, Komrokji RS. JAK Be Nimble: Reviewing the Development of JAK Inhibitors and JAK Inhibitor Combinations for Special Populations of Patients with Myelofibrosis. *J Immunother Prec Oncol*. Published online June 22, 2021. 2021;4:129–141.
4. Hussein SE, Wang SA, Pemmaraju N, et al. Chronic Myelomonocytic Leukemia: Hematopathology Perspective. *J Immunother Prec Oncol*. Published online June 18, 2021. 2021;4:142–149.
5. Tang K, Nastoupil LJ. Real-World Experiences of CAR T-Cell Therapy for Large B-Cell Lymphoma: How Similar Are They to the Prospective Studies? *J Immunother Prec Oncol*. 2021;4:150–159.
6. Soyfer EM, Fleischman AG. Inflammation in Myeloid Malignancies: From Bench to Bedside. *J Immunother Prec Oncol*. 2021;4:160–167.