THE UNIVERSITY OF TEXAS
MD Anderson Cancer Center®

IMPACT REPORT

June 2020

Sagerstrong Foundation

Naveen Pemmaraju, M.D.
Leukemia

HOW YOUR GIFT IS MAKING CANCER HISTORY®

Dear Mrs. Stacy Sager and Sagerstrong supporters,

It is with great appreciation and a deep sense of gratitude that I write to you. Your generous philanthropic support to my research fund has been critical to our accomplishments.

My clinical trials and research portfolio have remarkably increased over the past few years, in large part due to the generosity of donors like you. In the acute myeloid neoplasm (AML) and related neoplasms field, the major development in my career is that I led the clinical development of a novel agent, and we pioneered the first drug approved in a rare blood cancer: Tagraxofusp in blastic plasmacytoid dendritic cell neoplasm (BPDCN), which led to the first-ever approved CD123-targeted agent. This project was the realization of one of the most vital parts of my research, which is for patients with rare and ultra-rare blood cancers, as BPDCN, which was formerly considered as a rare subset of AML and now understood to represent its own myeloid neoplasm category, affects only 500-1,000 Americans per year (Pemmaraju et al NEJM April 2019). Furthermore, this was a national clinical trial, in partnership with pharmaceutical team members, academic investigators, not-for-profit entities and a network of diverse stakeholders that we helped to build, establish, and now must maintain, as we continue to investigate further combinations and novel therapies for patients with BPDCN.

In the AML translational field, our group is researching and characterizing a rare variant of a subset of AML known as APL, or acute promyelocytic leukemia, and we are on track to report and publish these novel findings in the literature with aim by 3rd quarter of this year.

In the myeloproliferative neoplasms (MPN) space, I have had the honor to lead a program with some exciting breakthroughs and leads. MPNs represent an important area for scientific consideration as there are few approved therapies

and a subset of patients with MPNs go on to subsequently develop AML. In the past year, we published our group's experience, the largest in the world, examining the incidence and outcomes of patients with lymphoma after having MPN and Janus Kinase (JAK) inhibitor therapy. This has become an emerging issue in our field, as our European colleagues published a widely reported paper, which hypothesized this would be a very common phenomenon. We also published a report, which was widely covered, that fortunately demonstrated this was actually a rare event in our large patient experience (Pemmaraju et al Blood 2019). The accompanying scientific editorial in *Blood* nicely captured the discussions and future questions in this field. Furthermore, it established my team, here at The University of Texas MD Anderson Cancer Center as world leaders.

We have multiple future directions that will require funding and support: examination of differences in European vs. American cohorts with this lymphoma incidence; the supposition that there may be a "pre-lymphoma" clone already existing in MPN patients that needs advanced pathology analysis with novel techniques; and finally, the identification of these "pre-lymphoma" clones in future, prospective studies. I am planning to direct portions of your funding towards launching this important initiative, which will represent a large-scale multi-disciplinary effort among pathology, our MPN team, statisticians and lymphoma colleagues.

In the MPN clinical trials space, I am happy to report that I am serving as Principal Investigator (PI) for several key clinical trials in our field. The LCL161 phase 2 clinical trial, solely started/ completed at
MD Anderson, just enrolled our 50th and final patient. This work has been presented at the ASH Annual Meeting over the last 3 years. This could open up a new area of research, one that we led — SMAC Mimetics in MPN patients, as we are showing an approximate 30% response rate in, primarily, high-risk patients. Furthermore, I recently led/reported interim/ongoing results for SL-401 (Tagraxofusp) in patients with MF and CMML, at ASCO 2019 Annual Meeting and EHA 2019 Congress. This study is ongoing, and of interest, as it is the same drug that I led to FDA approval for BPDCN. I am also our site PI for two other studies in MPN patients, these two combine Ruxolitinib with novel agents and are just opened with no publically available preliminary data yet. One important area I am planning to direct some of the funds you have provided me will be for adding novel aspects to these clinical trials, including add cytokine, lab correlative and bio-marker analysis; that typically do not carry funding sources but can often yield novel, ground-breaking preliminary scientific funding. So often, funding from government or industry is still lacking, that is why your support is so crucial; to help us to go for these type of elements.

Because of your generous support, you have given me and my team supreme flexibility and room to plan for novel studies for the next one-to-two years. The importance of this scientific "freedom" cannot be overstated. Lastly, as you know, we both have media and communications in common as something we respect as a powerful medium for dissemination of information. I have developed a novel patient survey/questionnaire online with Patient Power, one that already 400+ patients have filled out and we are currently in the analysis phase. I envision setting some of this fund aside, to help me further develop and add to this pilot study, which had little to no funding in its inception phase. It has been highly successful thus far; the next steps will be to add to the sample size, power and range of patients we approach.

I am in the process of planning a novel podcast/ YouTube grassroots program with a colleague to start a show discussing MPNs and rare blood cancers in a series of high-yield, straightforward chats, meant to be directed to the lay public, and which can be archived; freely available online. Because the series will not have any outside influence, it will reflect organic, grassroots, expert-driven programming and content. With your expertise, experience and your generous ongoing support, I believe we will be able to launch this effort via your fund and support by 2nd Quarter of 2020.

I believe that my passion and mission in life is to help raise awareness and develop novel therapies for patients with the most rare of blood cancers, such as MPNs. I believe that if someone has a rare

disease, it is not rare to them, their mother or their family; it is a disease; one that must be treated. I will not rest; I will strive to give a voice to those who do not have one, those who are devastated by having a diagnosis, which may affect very few people and ones that their physician may not be familiar with. Your support will greatly help me with my life's work and mission.