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Running Title: QOL and Communication in CML
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DISCLAIMER
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A recent publication in this journal has looked at a quality of life (QOL) survey in chronic myeloid leukemia (CML) patients and how decisions were made with their physicians (1). This evaluation of CML and other diseases has come to prominence over the last few years. With CML, the rationale is obvious. As I see it, the three main goals of CML therapy are survival, the ability to achieve a treatment-free remission (TFR) and avoidance or management of side effects. Survival for newly diagnosed CML patients is roughly the same as age matched controls, even when treated with first generation drugs (2). Newer drugs have perhaps made some cosmetic improvement here, although speed of response is improved. There is a likelihood as well that more patients become eligible for TFR attempts, although the success rate in appropriate patients is roughly the same regardless of the drug used to get there (3). Newer drugs are associated with different side effects, some less chronic, some potentially more severe, and many develop with time and are not seen early in studies that lead to product monograph information (4). Stating a drug is safer in the short term, can sometimes be replaced by more severe issues with longer term use. As we go from the stringency of a clinical trial to real world exposure, reports of new issues routinely appear.

So, we have roughly two thirds of new patients do not achieve successful TFR or have side effects on drug with or without a TFR attempt, especially if on therapy long term. They may very well have QOL issues. Here is where things get dicey. How do we define QOL (5)? To me, it is living a normal life without anything that has a negative impact. In the case of CML patients, QOL can be impacted by many things including issues unrelated to CML – other health issues, psychological or social issues, family issue, work issues, etc. Sorting these out may not be an easy task. For CML specifically, this can include side effects of therapy but also psychological aspects of diagnosis and ongoing therapy, impact of therapy on the ability to have normal family and interpersonal relationships including pregnancy, continue with work or education to name a few. Depending on where a patient live, this can include therapy availability, both drug and monitoring, and the elephant in the room – financial adversity. Newer drugs including in some cases generics, are not necessarily available, never less expensive and this can impact patients who do not have access, both in terms of their health and in the always present question, are they getting the best available therapy? It must be remembered as well that some of these concerns have a cultural or geographic basis. I fear that the side effect/adverse event issues are taking over the QOL discussion in the parts of the world with more comprehensive healthcare, as these can be the portal to suggesting management changes, especially to newer drugs. Some of the issues described in this paragraph are discussed in the referred to manuscript. At the recent CML Horizons global patient advocacy conference in Bucharest, it was very clearly stated by advocates from less advantaged countries, that their main issues are resources, geography and culture based all of which have significant QOL impact and believe that these issues must be addressed on a regional level.

So, this paper, as do most, has strengths and weaknesses, some of which are acknowledged by the respected authors. The most significant strengths that cannot be disputed are 1) QOL, but with varying definition, is important to patients, and 2) communication with treating physicians is an issue. The weaknesses related to a “one size fits all” QOL definition have been discussed above.

Things change with disease status and side effects and there is always a balance about what can be tolerated. Those of us who have treated CML over the decades have always seen how some patients do not complain until an alternative to their current management materializes. All patients in this study were on at least their second TKI. Why the switch – resistance, desire for better response, intolerance, suggestion of fewer side effects? With the suggestion of “failure” of earlier therapy, this will potentially color expectations. Given the reimbursement options available, some of these patients may be locked in to current therapy. No data on “successful” first line patients is given as a comparison.

Well-meaning patient advocates and physicians who believe that they can speak for the world are represented here. Unmet needs and communication have resource, geographical and cultural bases just like QOL (6). Patient expectations vary based on education, access to unbiased information and the

reality of what are available. These data are also retrospective. At the time of decision making, what were the expectations? I daresay that this may be different in retrospect especially with "failed" attempts. How were patients and doctors chosen here and by whom? There is at least bias in that they came from countries with developed health care systems. Without wanting to give the impression that I am trying to put down involved patients or more general hematologists, much of the information that forms their basis has a degree of filtering even with published studies, leading to conclusions that may not be applicable to an individual case.

Doctors can be quite paternalistic, and this can be due to ego, culture, patient education and understanding. This is probably more of an issue in the patients not represented here. Also the knowledge base of the patients in this study has likely improved from the time since diagnosis and things that could have been discussed were not initially obvious. The type and source and practicality of new information can also contribute to this. "You have leukemia" answered with "I want to be cured" is the immediate first conversation. A patient may be too shocked, not knowing what to ask and the doctor, giving them credit for being open-minded, may be unaware of what is additionally needed. Some patients simply do not want to know more. Of course, this should be an ongoing dialogue. And as the disease evolves, life goes on, other pressures come into play, goals of therapy may very well change, again part of the ongoing dialogue. The patients represented here have had considerable time to think about how their particular journey has progressed and all of us can always second guess ourselves about how we could have done things better.

I hope this study can serve as a basis for improving patient care. This is the beginning, not the end product. We need to educate patients be more open with their physicians about issues beyond just side effects and for their physicians to be more receptive with offering discussion and solutions (7). This must take into account regional differences. Data must be collected to encompass all disparities. Physicians practicing in all regions must be included. And finally, although we are all eternally grateful for the support of pharma in drug development, research and patient support and advocacy, studies like this must be at arm's length.

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