The Patient Perspective on TFR

Pat Elliott
CML Patient and Patient Advocate
United States
CML Patient Environment

- Gaps in basic CML understanding
- Multiple treatments = CML community confusion
- Side effects/quality of life perspective differences
- Response vs remission vs TFR often unclear
- Lack of “patient friendly” TFR information
- TFR underway in and out of trials
- Formal and informal TFR knowledge differs
- Bottom line: TFR not well understood, much confusion as well as hope, excitement
TFR Patient Environment

- Unaware of/not following global trial data
- Learning from anecdotal data – patient groups
- Key data often left out of patient conversations:
  - Importance of adherence in obtaining durable, stable CMR
  - Durable stable CMR before discontinuation is important to limit molecular relapse
  - Success rate - Trial data indicates in select patients some 40-50% may be able to cease therapy
  - Success factors include prior IFN therapy before TKI therapy, longer duration of CMR before discontinuation and longer duration of Imatinib use before discontinuation
  - Unknown if second-generation TKIs result in an increased probability of TFR
Facebook group for TFR patients – 70 members
Primary support resource for these patients
TFR Patient Survey  April 2015

- 50 responses, 36 in TFR
- Age range: 18 (dx age 12) - to 71 (dx age 61)
- From: UK, France, Ireland, Scotland, South Africa, Australia, United States
- Most not in clinical trials, work closely with hem/onc
- Longest TFR – Six years - Male, South Africa, 70, dx 2003, Interferon, Gleevec, Stopped treatment 2009, Undetectable, Feels “great with no side effects.”
Survey: Key Reasons Seeking TFR

- 74% - Relief from side effects
- 74% - Concern about long term effects
- 13% - Costs of treatment

“Had no quality of life due to severe side effects.”
“Taken off for side effects, chose not to return.”
“Stopped two months before telling doc. Great difficulty getting consultants to believe how very bad things were. It's my body and mind, I have the final say.”
Physical Impact of TFR – Clinical*

- Clinical trial data: muscle, joint, bone pain, sweating, skin problems, depression, fatigue
- Grade 4, Characterized as “minor” side effects
- Late relapses = need for long term monitoring
- Protocols to prevent/manage “withdrawal symptoms” and emotional impact are not clear
- Protocols for long term management of TFR patient needs are not clear

*European Stop Tyrosine Kinase Inhibitor study (EURO-SKI) Interim Analysis - 200 of 700 patients
Physical Impact of TFR – Patient

Mixed responses – positive and negative

- Cessation side effects worse than on drug.
- Joint pain/cardiac issues worsened, required additional medical support.
- Side effects less; low level joint pain remains.
- Side effects and fatigue lessened with time.
- No side effects. Feel really good.
- Turned down trial offer - Patients report bone pain lasting weeks to years, need to manage with another drug, this is not “treatment free.”
Mixed Responses

- Needs to be defined more clearly. Studies from Europe show patients who “re-start” do not “fail.”
- It’s not failing, its just another step in the process of this dreaded disease.
- The word 'devastated' comes to mind. The drugs have done so much damage, to have to restart them is nearly as bad as a death sentence.
Survey: Psychological Concerns

- **Isolation**: Want to talk to other patients in TFR
- **Response loss**: If lose remission, may not be able to regain response at same level as before TFR.
- **Uncertain Future**: Not clear what’s next. Developed unexpected clinical issues after going treatment free.
- **Side effect support lacking**: Make health professionals aware patients may have side effect issues for life. Going off TKIs does not mean the end of CML for a lot of patients but a different phase of the cancer that needs be dealt with in a unique and challenging way.
Survey: Emotional Support

**Majority view** - Psychological support is crucial as treatment cessation does not work for everyone.
- Emotional support is needed, it’s a difficult journey, very new and unknown.
- No real support was offered by my treating doctor.
- My family has and continues to be my constant support. No other avenue was offered or available.

**Minority view** – Just another step in CML journey, no emotional support required.
Survey: Medical Community

- “Need to give serious consideration to the dreadful side effects that can be debilitating and not focus solely on bcr/abl results.”
- “More support is needed, starting with the medical profession. I don't think medical profession realizes how stressful not being treated is.”
- “Include pediatric patients in TFR clinical trials.”
- Recognize “personalized” nature of TFR response, patient level of side effects, emotional need varies.
- Need guidelines, consistencies – i.e. PCR test schedules range from monthly to every 6 months
Personal TFR Experience

- 09/09 CML diagnosis, second cancer
- 09/10 Learned about Stop Imatinib (STIM) trial
- TFR primary goal, strict adherence, followed studies
- 03/11 forward, undetectable, MR 4.5
- 04/13 – Clinical trial wait list
- 11/13 – Hem/Onc refused personal TFR trial
- 04/14 – Personal TFR trial with new hem/onc
- Lost weight, lost edema, gained energy, brain fog lifted, no adverse side effects, felt great
Detectable at 6 & 10 week PCR testing
- Oncologist opinion conflicted with trial data
- Got second opinion from Dr. Brian Druker
- 08/14 - Resumed treatment, second line therapy to obtain deeper response
- 09/14 - Stopped treatment due to pancreatitis
- 09/14 – Restarted treatment at reduced dose and started weekly blood monitoring
- 12/14 – PCR 0.0024
- 03/15 – Undetectable 0.0000
- 04/14 – 03/15 = One year effort, will try again
Recognize the TFR patient journey is different and some or all phases may require emotional support:
- Decision phase before stopping
- Identifying clinical trial or physician with TFR expertise
- Cessation "probation period" with close monitoring and need for side effect and/or withdrawal symptoms support
- Potential for TFR failure and need to resume treatment
- Potential success, with need for continual monitoring
- Potential for relapse after long term TFR success

Recognize the isolation felt by TFR patients may be different from that of non-TFR patients.
Advocate Recommendations

- Review CML 101 information and programs so that TFR education is provided in accurate, patient friendly language.
- Be clear that treatment cessation may not result in complete absence of side effects and that long term monitoring is needed.
- Seek out and post information on TFR clinical trials – be proactive – don’t rely on medical community.
- Recognize that many view clinical trials as “experimental” and will have fears, concerns.
Advocate Recommendations

- Share information from reliable resources.
- Develop local information, support resources.
  - Example: NCMLS and LLS Patient Workshop in Phoenix 09/14 when TFR clinical trials opened
- Identify resources for patients who need emotional support.
- CMLA - Collect data from patients both in and out of trials to obtain comprehensive data.
- CMLA - Consider global, central online TFR patient information portal and support group.
Patients have the most to gain – and lose – from new treatments and stopping treatment.

Our voice needs to be included – evaluating not only clinical response, but also the impact on our quality of life and on removing obstacles that prevent access to treatment and support.

YOU can be one of those voices!
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