

Vesely, Nicole

From:

Sent: Friday, May 09, 2008 12:28 PM

To: Vesely, Nicole

Follow Up Flag: Follow up

Flag Status: Purple

DEAR DR. VESELY : I BELIEVE THAT I HAVE EMAILED YOU BEFORE CONCERNING ITP AND NEW DRUGS FOR TESTING. I BELIEVE THAT AS A PARENT OF A YOUTH W/ ITP IT IS IMPORTANT TO GET TRIAL MEDS. FOR TESTING ASAP, THERE ARE NOT A LOT OF GOOD CHOICES FOR TREATMENT WHEN FAILS USING STANDARD DRUGS. I AM FOR TESTING AS SOON AS NEW DRUGS BECOME AVAILABLE. THANK YOU. SINCERELY,

5/12/2008

Vesely, Nicole

From:

Sent: Friday, May 09, 2008 5:13 PM

To: Vesely, Nicole

Subject: FDA approval

Please accept this as a written correspondence concerning approval of thrombopoietin stimulators.

As a private practice hematologist, I strongly support the approval of thrombopoietin stimulators for treatment of chronic ITP. I currently have a patient who could potentially benefit from one of these agents. I have no financial interests in either of the pharmaceutical companies attempting to gain FDA approval. Please expedite the approval process so that these treatment options may become available.

Sincerely,

Matthew Beldner

--

Matthew Beldner, MD
Lowcountry Hematology & Oncology, PA
900 Bowman Rd, Suite 103
Mt. Pleasant, South Carolina, 29464
ph (843) 881-5844
fax (843) 881-9499

Vesely, Nicole

From:

Sent: Saturday, May 10, 2008 4:14 PM

To: Vesely, Nicole

Subject: Regarding The Platelet With hope For Cure

Nicole Vesely@fda.hhs.gov.

I am writing to tell that I am desperate for help with the ITP problem that was discovered in me in early in 2007. Other medical treatments and exploratory examinations into my heart area could not be done because I was discovered to have a low platelet count that forbid this procedure. Thus, I had to see a hematologist about my blood condition. What was learned from these visits was that there was nothing that could be done to improve my immune system or raise the low platelet number to a number of 200,000 required for a catheterization procedure to start. The count of concern was at 3000 on February 5, 2007. Nothing, of all the medicines tried, worked to jump the critical number. In April, the doctors decided to do the catheterization with the number I had, which was about 20,000 at the time. They found my heart arteries were satisfactory and I was told that the little blockage I had could be controlled with present drugs. The scare of bleeding to death kept me in the control of this doctoring group and after a full year of constant, weekly visits, it now appears while not bluntly said, that I am on my own to discover what might help me grow some helpful platelet cells. That there is little they can do except test me once a week to monitor my numbers. Naturally, I am disheartened with the medical treatment given to me. To be hospitalized for 12 days and then after all their experimentation, to be turned loose to my own choices and ministrations, is just unimaginable and hard to accept. There must be thousands of people like me being treated in a similar poor fashion and I would like to see a drug come to the market that is going to stop this blood disease from occurring and halting it when it does. I could name the things in me that do not look right and they might be related to this ITP problem but nobody knows. Example: Loss of hair on arms, legs, chest, and head. Pimples that are white or brown are seen and felt on my neck and back and arms. Need for more sleep, lack of endurance and strength, and failing eye sight might all be connected to the power of the Platelets, and my hope is that my letter helps find a cure for all these troubles. Thanks for asking and carrying the ball.

5/12/2008

Vesely, Nicole

From:

Sent: Saturday, May 10, 2008 3:39 PM

To: Vesely, Nicole

Cc:

Subject: support comments, re: Eltrombopag approval

Hello Nicole,

My name is _____, understand that Eltrombopag is coming up for FDA review/approval soon and wish I could attend the meeting to voice my support of the drug but I really can't attend in person to voice my support of the approval of this drug.

I am 57 years old and was diagnosed with ITP about 2 years ago. My platelet counts prior to participation in the GSK trials were in the 1,000-5,000 range. At the time I was initially diagnosed my platelet count was "1" (meaning 1,000).

I have been taking Eltrombopag daily since December of 2006 and have been under the medical supervision of _____

Following my initial diagnosis of ITP I received all the conventional treatments: Rituxin, Splenectomy, Steroids and Intravenous immunoglobulin. None of these drugs and therapies worked for me and my counts remained very low. My ITP was diagnosed as highly refractory.

In December of 2006 I started taking Eltrombopag as a study subject in GSK's RAISE trial. Since the start of the study I have not had the need for nor have I received any other therapies or drugs for the treatment of my ITP. Just prior to my starting the Eltrombopag clinical trial I required an IVIG transfusion every 2 weeks to keep my platelet counts above 50K.

I have responded well to Eltrombopag. My counts have been stable in the 70,000-100,000 range for the past year. I don't have to live in fear of getting a severe internal bleed and I don't have to severely limit my physical activities. I can roller skate with my 9 year old daughter without fear of getting a bad bruise. I don't feel extremely tired and washed out as I did when my platelet counts were very low.

I am sure it has made my insurance company very happy as well. In 2006, the medical costs to treat my ITP were around \$150,000, mostly due to the cost of the regular infusions. I have been taking 75mg of Eltrombopag once a day, without any side effects what-so-ever for over 1 year and it has kept my platelet counts in the "safe" zone for the entire time I have participated in the trial.

I can't imagine why this drug would not be approved by the FDA. At least in my case, it has been a life changing remedy to my condition and I am sure that there are a lot of people in the world who could benefit from the FDA's approval of this drug.

Please let me know if there is anything more I can do or say in support of having the FDA approve Eltrombopag.

Yours truly,

5/12/2008

Vesely, Nicole

From:
Sent: Friday, May 09, 2008 5:16 PM
To: Vesely, Nicole
Subject: Eltrombopag
Follow Up Flag: Follow up
Flag Status: Purple

Dear Nicole,
I think I wrote to you before regarding Amgen 531.

I have had ITP for 63 years. I had a splenectomy at age 11. since then I have had a stormy course of ups and downs with platelets getting as low as 2000. I cannot take steroids and the only treatment that helps temporarily is IVIG which is, as you know, expensive and lengthy infusion. It would give me great peace of mind to have a pill that would help solve this problem.

Please plead our cause to the FDA.

Sincerely,

Vesely, Nicole

From: Miranda Kolbe [mkolbe@schubertlawfirm.com]
Sent: Friday, May 09, 2008 4:53 PM
To: Vesely, Nicole
Subject: PROMACTA trial
Follow Up Flag: Follow up
Flag Status: Purple

Dear Ms. Vesely:

I am writing to provide my input regarding a proposed trial of PROMACTA, a drug developed by GlaxoSmithKline. The description of the trial, set forth below, indicates that the drug would be used “for the short-term treatment” of individuals with chronic ITP:

The committee will discuss the new drug application (NDA) 022-291, proposed trade name PROMACTA (eltrombopag olamine), by GlaxoSmithKline, proposed indication for the short-term treatment of previously-treated patients with chronic idiopathic thrombocytopenic purpura (ITP) to increase platelet counts and reduce or prevent bleeding.

My concern relates to the safety of the treatment. It seems that, at best, PROMACTA will effect a temporary remission from ITP. My understanding is that the drug will accelerate platelet production in the short-term only, and will have no effect on platelet production in the long-term. In addition, the drug will have no effect, in either the short- or long-term, on the platelet destruction that endemic to the disease. Therefore, when individuals with ITP stop taking the PROMACTA, their ITP will almost certainly recur. As a result, the only real utility of PROMACTA to people with chronic ITP will result from long-term, not short-term, use of PROMACTA.

Assuming that there have not been studies of the safety of PROMACTA when used in the long-term, it seems unethical to authorize the proposed clinical trial of this drug.

I would appreciate any feedback that you may have to my input.

Thank you,

Miranda Kolbe
Schubert & Reed LLP
Three Embarcadero Center, Suite 1650
San Francisco, CA 94111
Telephone: (415) 788-4220
Facsimile: (415) 788-0161

5/12/2008

Vesely, Nicole

From:
Sent: Friday, May 09, 2008 12:55 PM
To: Vesely, Nicole
Cc: pdsa@pdsa.org
Subject: Promacta; Platelet Disorders
Follow Up Flag: Follow up
Flag Status: Purple

Dear Ms. Vesely:

As someone who suffers with low platelet counts, I am writing to you in order to request that you do whatever can be done in order to cause the FDA to expedite their approval of Promacta, or any other drug in their pipeline that brings relief and hope to those of us who suffer with low platelet counts.

Sincerely,

5/12/2008

Vesely, Nicole

From:

Sent: Monday, May 12, 2008 10:17 AM

To: Vesely, Nicole

Subject: Promacta

Ms. Vesely,

I write this email with hope that Promacta could be the drug that actually could save lives and help those like me, with acute ITP. I have been on a "roller coaster" for almost 3 years. I've always been very healthy and found out 3 years ago that my platelets were at 90,000. They then dipped to about 55,000 and stayed there for about 1 1/2 years. I was okay with that because I very rarely had to take prednisone. Last November my platelets dropped to 18,000 and even with doses of prednisone would not keep from dropping. I was admitted into the hospital in February, 2008 for an IVIG treatment (of which I responded well to) and my platelets have since dropped, but have remained above 40,000 for almost 3 months.

Promacta would be a wonder drug for people like me. If the FDA can pass it, it would definitely improve the quality of life for many people. In many cases, it might even save lives. Please consider this drug.

Thank you.

5/12/2008

Vesely, Nicole

From:

Sent: Tuesday, May 13, 2008 12:39 AM

To: Vesely, Nicole

Cc: pdsa@pdsa.org

Subject: Re: Your input wanted for an important FDA meeting on May 30

Thank you very much for your mail dated 9th May, 2008. I, _____ aged 50 years is resident of _____) and am pure vegetarian. Since my childhood, I had been in good health except High Blood Pressure since last 3-4 years which is totally under control with mild medication. I have 2 children both boys aged 27 years and 25 years. I have still to achieve Menopause though with irregular menstruation. I have Hb of about 12. In 2002, I was to be operated for Polyps (Nose) and a test of Platelet count was conducted for that purpose. Though occasionally, my nose bled, but I was told by ENT Specialists that it was due to disturbance caused in tiny blood vessels only and 1st time it was revealed that I was having low platelet count of 35,000 only (normal range 1,50,000 to 4,00,000) and the operation was postponed for want of increase in platelet counts to a minimum level of 1 to 1.5 lakhs. Series of test including bone marrow concluded that I was suffering from ITP. I was put on steroids and I responded well to the medicine and my counts increased to a level of 1.5 lakhs and I was successfully operated after 1 month time. The steroids continued but after some time the platelet counts started to deteriorate and after 1 year or so, it was in the range of 40,000 to 60,000 only. Since steroids were affecting the body adversely, the same were discontinued with the advise of the medical practitioner (Head of Hematology Deptt.) _____ I also tried Homeopath and Ayurved medicines which showed no sign of improvement. Since last 3 years, I am not taking any medicine except body immunity system enhancer like calcium, vitamin etc. and since then my platelet counts are ranging in between 15000 to 30000 only. Medical practitioners say that there is no further test to know the reasons of low platelet count and they are not sure whether removal of spleen will set the problem right?

I look forward to your appropriate and useful advise in this regard.

With personal regards,

On Fri, 09 May 2008 The Platelet Disorder Support Association wrote :

>

>-----

>-----

>

>Your Input Wanted for an Important FDA Meeting on May 30

>

>

>Rockville, MD, May 9, 2008- In order for new treatments to be available for ITP

>they need to be approved by the Food and Drug Administration (FDA). Many of you

5/13/2008

May 12, 2007

Ms. Nicole Vesely
Center for Drug Evaluation & Research (HFD-21)
Food & Drug Administration
5600 Fishers Lane
Rockville, MD 20857

Dear Ms. Vesely,

I am writing to urge the FDA to go ahead with approvals for NDA 022-291, or Promacta from GlaxoSmithKline for treatment of persons with idiopathic thrombocytopenia purpura (ITP).

So many of the drugs that are available either no longer work for patients, or have caused more damage to their bodies than care to deal with. Imagine a 34-year old woman finding out that she has severe osteoporosis due to long term corticosteroid use. This severe osteoporosis is worse than her 68-year old mother's osteoporosis. Corticosteroids are one of the few treatments that have worked for her, and she's had to be on them for over 6-years so far. This causes further medical problems such as bone breaks, which of course lead to more expenses in an already overly expensive medical system.

Many patients would prefer not to take the highly toxic chemotherapy type drugs, because of the potential damage those can cause; nor do they want to go the route of surgery because there's no guarantee of it working.

Promacta, and other thrombopoietin (TPO)-like treatments are a ray of hope for every patient that has endured the miserable rollercoaster ride that is ITP, and it's time for something new.

As an ITP patient, I urge the FDA to go ahead and approve this NDA. The sooner the better.

Thank you for your time.

Regards,

Vesely, Nicole

From:

Sent: Monday, May 12, 2008 4:48 PM

To: Vesely, Nicole

Subject: surviving ITP

I am a 54 year old woman with ITP. I was diagnosed in the summer of 2005 when presurgical bloodwork was being done for an upcoming hysterectomy. My platelets were 1,000, and my doctor referred me immediately to an oncologist. I was placed on steroid treatment and continued to worsen. I was hospitalized and given platelets and blood transfusions to stabilize me, and had an emergency hysterectomy. All further treatments were ineffective and the side effects were horrendous! My platelets continued to fall, in spite of multiple therapies, and the decision was made to do a splenectomy four months later. It has been a year and a half since the splenectomy. My husband lost his job and we lost our health insurance, so I haven't been to a doctor since then. I have not experienced petechia, or serious bruising, but any cuts do not stop bleeding as quickly as they did pre-ITP. I do worry that if I should relapse there's no treatment left to save my life. I am fairly healthy and active in general and hope to live a full lifespan. I would be happy to provide you with further information, if you wish. I would be grateful if you could address my concerns. I'm sure there are others in my position, as well.

Sincerely,

Vesely, Nicole

From: .
Sent: Monday, May 12, 2008 3:37 PM
To: Vesely, Nicole
Subject: Promacta and ITP

Dear Ms Vesely,

My daughter, age 3, has recently been diagnosed with Chronic ITP, although we have been living with the condition for over a year and she is the only case in with chronic ITP. If there is any truth in the results for the above chemical treatment or maybe if it can give us even the slightest piece of hope that there is something that will help (i am not even asking for a cure) or make these peoples and their carers lives manageable. Who ever the regulatory authorities are, they should allow this to become accessible to all ITP sufferers.

With this you have my full support here from

Kind regards,

Yours sincerely,

Get news, entertainment and everything you care about at Live.com. [Check it out!](#)

Vesely, Nicole

From:
Sent: Wednesday, May 14, 2008 9:59 AM
To: Vesely, Nicole
Subject: FDA Meeting Input
Follow Up Flag: Follow up
Flag Status: Red

Good Morning

My mother has been diagnosed with ITP and it has been a battle. The Prednesone makes the bruising go away but her platelets seem to stay low. I am presently looking for a second opinion. She has had blood transfusions and nothing seems to help. Her doctor wants to have her Spleen removed – I'm not sure about that. So it's wait and see right now. I am in contact with _____ and I am looking for a specialist in _____. It's sad to see someone you love go through this – medication is not working. I would like more information on what comes from this meeting.

Anything would be helpful at this time.

I appreciate all the research and medication that may come from that research.

5/14/2008

Vesely, Nicole

From:

Sent: Wednesday, May 14, 2008 8:06 PM

To: Vesely, Nicole

Subject: Meeting of May 30th

I support the drug if it can help us with ITP to make more platelets as of now nothing I have tried has help me to stay in a save range with my platelets. Any help the FDA can give us I would appreciate, Please add my name to the list of people who support this new drug.

Wondering what's for Dinner Tonight? [Get new twists on family favorites at AOL Food.](#)

5/15/2008

Vesely, Nicole

From:

Sent: Monday, May 19, 2008 4:44 AM

To: Vesely, Nicole

Hello, my name is [REDACTED], I have suffered since the age of six with I.T.P. I am now 35 years old. I have tried every treatment known and still have not been able to keep my platelets above 4,000. There has been many times I almost died. I have had to quit many jobs because I was not able to keep up with the rest. I have known this illness all my life. I can honestly say I do not know what it feels like to feel and be healthy. I would love a try at this new drug! I have heard so much about it through others. I am pretty much at the point where they can not do anything for me. I want to live the rest of my life knowing and being healthy. This drug may be just what I and so many like me need. A chance to live a life we have never had or in some, lost. Please help us! In some of our cases, we have nothing to lose.

thank you,

--

5/19/2008

Vesely, Nicole

From:

Sent: Sunday, May 18, 2008 4:01 AM

To: Vesely, Nicole

Subject: Input for FDA meeting on May 30

Hello Ms. Vesely,

My name is [redacted] I am a doctoral student in the school of engineering at the [redacted] The reason I write to you, is because I suffer from ITP - Idiopathic Thrombocytopenic Perpura - and wish to share my hope for a timely (quick) approval of T-Po therapies which could help fight this disease.

I am 24 years of age, very passionate about science and maths, very interested in innovation, and the recipient of a special research fellowship at my university.

Last year, my doctors found my platelets to be so low that they put me on a heavy dose of steroids, and then immunoglobulins. What ensued was a painful couple of months with sleepless nights, inability to focus, and constant anxiety. When those therapies did not work, the doctors tried other combinations of approaches to suppress my immune system further. A month later, my spleen was removed in a major surgery.

Nothing improved, but each treatment was dangerous to my immune system. It seems from discussions with my doctors that the approach for therapy should have been NOT to suppress my immune system, but to enhance the production of platelets in my blood. That requires a T-Po drug, which the FDA has not yet approved.

I am a researcher myself, in addition to having been a patient, so I understand the critical importance timely availability of research, especially drugs in a disease like ITP, where a comprehensive treatment is still not available. The only line of therapy which the doctors use on ITP patients is suppression of immune system, which can be just as bad as the disease. T-Po drugs present a much more hopeful approach - one that enhances growth of platelets that we need, instead of suppressing the immune system.

For me, each day is a bigger challenge - I go to the lab, and it's not just the research that's on my mind, it is also a slow countdown of how many days I have left before I have fatal inner bleeding.

My platelets are 2000, which means I could bleed to death any moment. The only treatment approach which the doctors have is to suppress my immune system even further, which is deadly itself. Like many ITP patients, and as a young scientist with dreams and aspirations, I await the approval of T-Po drugs and funding of research in blood disorders especially ITP.

Thanks,

P.S. - Would have been very happy to come and present my views in person, but with a student, affordability of travel is always a consideration.

--

5/19/2008

Vesely, Nicole

From:

Sent: Friday, May 16, 2008 6:01 PM

To: Vesely, Nicole

Subject: FDA approval of N-PLATE

Dear Nicole,

My name is [REDACTED]. My son [REDACTED], now to turn 7 in June, IS IN NEED of N-PLATE. This has been the 4th time he has had an episode of ITP. Which by the way has now become chronic. He goes between 0 and 33 thousand (has of May 9th). Which has been the highest. He's in 1st grade and missed about a month and a half of school since Nov. IVIG didn't work this time, neither did steroids (but he gained 15 or so lbs) at 80mg a day, and then we did rituxan treatments in Jan. To no avail, nothing has worked this time [REDACTED] tried to get him in the study, and unfortunately it was closed to age group. So we've been waiting patiently for this drug to be available. I don't know what else to do but beg on my child's behalf. I know if anyone at the FDA had a child with rare disorder, that they would be helping it to go through faster. Since all other treatments affect the immune system, whereas N-PLATE works quite differently. Please keep me, and all the others who really need this drug, informed as soon as the FDA has decided when they will release it for us to start treatment. Thank you for helping us and doing what you can!

Sincerely,

5/19/2008

May 16, 2008

Nicole Vesely
Center for Drug Evaluation and Research (HFD-21)
Food and Drug Administration
5630 Fishers Lane, rm. 1093
Rockville, MD 20857

Dear Ms Vesely,

Re: Promacta (eltrombopag)

I am writing to you to express my support for the FDA approval of Promacta (Eltrombopag). I am a 47 year old male who prior to January 2007 was in perfect health.

My ITP (Immune Thrombocytopenic Purpura) symptoms began in January 2007 when I began experiencing numerous, uncontrollable nose bleeds, large bruises and petechiae throughout my body. I was immediately admitted to the hospital in [redacted] because my blood platelet count was 2,000 (normal is 150,000 to 400,000). During my first three-day stay, several tests were performed on me including a bone marrow sample. After receiving three treatments of IVIG and heavy dosages of steroids, I was released from the hospital when my count was at 30,000.

For the next three weeks, I went to the Oncology doctor twice a week for a platelet count to monitor my platelet counts. All of this time I was taking 80mg of Prednisone which was keeping me up at night, making me irritated and causing me to gain weight. The doctors kept asking me if I selected a surgeon yet to remove my spleen. I told them that I thought that is was too soon and would rather explore other treatments first. They informed me that there are no specific treatments for ITP and started me on four treatments of Rituxin. During this time, my count dropped to 12k and I was again admitted into the hospital for three days of IVIG and Rituxin.

During my stay in the hospital, I did research on the internet to find an expert in ITP so that I could obtain a second opinion. I found a doctor at [redacted] but he was not available for several weeks. I continued to go to my [redacted] doctors twice a week for platelet counts and continued the Rituxin treatments and IVIG.

I finally visited the [redacted] doctor and he treated me for H.pylori and recommended to stay on a daily dosage of 80mg of Prednisone.

During that time, I also found a wonderful source of information on the internet called the PDSA (Platelet Disorder Support Organization). The material, personal contacts, web blogs and stories have been an extremely useful tool for my family and me. It is a shame my [redacted] doctors were not familiar with the organization since they may have changed their treatments.

After a few more months of the same treatment with the [redacted] doctors, I was put in touch with an ITP specialist from [redacted]. He immediately put me on an eltrombopag study and I saw positive results right away. The study was three cycles of six weeks on 75mg/day and 4 weeks off while trying to taper off the Prednisone. As I approached the end of each six-week cycle, my count was 60 to 70k. Once I got off the eltrombopag, my counts

immediately dropped to 20k and I frequently got nose bleeds and bruises. By the end of the study, I was down to 20 mg/day of Prednisone and had steady counts of 60 to 70k.

I began the extended study in February of this year with a daily dosage of 75mg/day and no more Prednisone and my counts have been steady at 69 (April 18th count).

Thanks to Eltrombopag, I feel like I finally got my life back. I am back to doing the things that I enjoyed prior to being diagnosed with ITP. I skied seven times this past snow season as opposed to zero times last year. I can ride Quads/ATV's in the woods with my family which I could not do last year for fear of what may happen to me if I got hurt. I am back to playing basketball with my daughter since last year every time I played, my hands and arms ended up bruised. I lost the 21 pounds I gained while on Prednisone and I am no longer cranky with my family and friends since I now feel confident that the treatment that I am taking is showing positive results. And I finally feel that I no longer have to stress out about not selecting a surgeon to remove my spleen.

My goal in writing this letter is to give you a real-life case study about the frustration that my family and I had to endure for the past 17 months due to the lack of available ITP treatments and medical knowledge and to tell you that I am a living proof that I have benefited from the Eltrombopag treatments. I am asking the FDA to expedite the medical release of this drug so that other patients with ITP could have an available treatment that will enable them to regain their lives back just like I did.

Sincerely,