Transcript Details
This is a transcript of an educational program. Details about the program and additional media formats for the program are accessible by visiting: https://reachmd.com/programs/medical-industry-feature/adult-chronic-immune-thrombocytopenia-management/56537/

ReachMD
www.reachmd.com
info@reachmd.com
(866) 423-7849

Identifying and Managing Adult Patients with Chronic Immune Thrombocytopenia

Announcer:
This is a promotional video funded by Sobi. The healthcare professionals in this video have been compensated by Sobi for these discussions- the views expressed during the video may reflect the personal opinions and experiences of the healthcare professionals. Any patient cases discussed are not representative of every patient's treatment journey, individual results may vary.

Doptelet is indicated for treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia, who have had an insufficient response to a previous treatment.

Please see Doptelet important safety information in this video and full Prescribing Information at www.Doptelet.com.

IMPORTANT SAFETY INFORMATION

WARNINGS and PRECAUTIONS

Thrombotic/Thromboembolic Complications
DOPTELET has been associated with thrombotic and thromboembolic complications in patients with chronic ITP. In adult patients with chronic immune thrombocytopenia, thromboembolic events (arterial or venous) occurred in 7% (9/128) of patients receiving DOPTELET.

Consider the potential increased thrombotic risk when administering DOPTELET to patients with known risk factors for thromboembolism, including genetic prothrombotic conditions and acquired risk factors.

DOPTELET should not be administered to patients with ITP in an attempt to normalize platelet counts. Monitor platelet counts and follow the dosing guidelines to achieve target platelet counts. Monitor patients receiving DOPTELET for signs and symptoms of thromboembolic events and institute treatment promptly.

Please see additional important safety information throughout this video.

Shawn Streeter:
Hello, everyone. I hope you're doing well today. My name is Shawn Streeter. Today's discussion topic will be identifying and managing adult patients with chronic immune thrombocytopenia. I have with me today, Doctor Panch, an expert in the field of ITP. Doctor Panch, would you like to introduce yourself?

Dr Panch:
Thank you Shawn. And thank you, everyone. I am Sandhya Panch, haematologist from the University of Washington, Fred Hutch Cancer Centre. I'm also director for transfusion services at the Fred Hutch Cancer Centre, and I have the privilege of treating patients with ITP and autoimmune cytopenias.

Shawn Streeter:
Thank you for being here with us today. Would you be able to give us a brief overview of chronic immune thrombocytopenia?

Dr Panch:

Yeah. So ITP as you know, is an acquired immune disorder where platelet counts are typically less than 100,000 per microliter. And these low counts have to persist for more than a year to be termed chronic. Currently, ITP affects approximately ten per 100,000 adults in the United States, and it is more common in young women compared to young men, although this proportion evens out over the age of 65 years.

Shawn Streeter:
And what can you share about the pathophysiology of this disease?

Dr Panch:
ITP is a complex autoimmune disorder. The precise mechanism of disease is unclear, but we know that there are two key processes at work. Autoantibodies target megakaryocytes, the precursor cells that produce platelets, and this reduces the number of platelets produced by the body. And healthy platelets are targeted by autoantibodies, resulting in their destruction by macrophages. So the platelet deficit in ITP is caused by two pathways: inhibition of platelet production and accelerated platelet destruction.

Shawn Streeter:
So you mentioned a key phrase there, Doctor Panch. Platelet deficit. So what does this platelet deficit mean for patients? How is the patient affected by ITP and what are the consequences?

Dr Panch:
So common symptoms include bleeding, bruising and fatigue. Chronic ITP carries an elevated risk for bleeding and infection and mortality.

Negative impact on the patient's physical, psychological, and health related quality of life occurs as well. We see this in the results of the ITP World Impact Survey or the I-WISh. The I-WISh study was a cross-sectional survey, which included 1507 patients and 472 physicians, with an aim to establish the impact of ITP on health related quality of life and productivity from patient and physician perspectives. And the survey results showed a high proportion of patients reported limited ability to perform daily tasks, also reported impaired social life, and a reduction in energy levels.

Shawn Streeter:
So how do you decide the optimal treatment approach for an individual patient?

Dr Panch:
There are several ways to treat ITP, Shawn. Every patient is different, not only in terms of their clinical presentation, but also in their preferences and treatment goals.

When selecting a treatment that will suit the patient, the shared decision making approach is recommended. With this approach, the patient is educated and empowered to make these decisions as a partner with the physician.

While the platelet count is a goal of therapy for ITP, when choosing a treatment, other factors are important to consider. For example, the patient's treatment goals, the patient's preferences, which include you know route of administration of the drug, monitoring requirements, their dietary needs, as well as social determinants such as social determinants of health like age and financial stability, health literacy, proximity to facilities, and a support system.

Shawn Streeter:
So it sounds like there are a few important considerations to make. What therapy options are there for patients?

Dr Panch:
Most patients with ITP are initially treated with a corticosteroid. Second line options include rituximab, splenectomy, thrombopoietin receptor agonists, or TPO-RAs. Each second line option has its own pros and cons, and the patient values and preferences should be considered and discussed.

Shawn Streeter:
And would you be able to describe a typical scenario involving treatment selection for a patient?

Dr Panch:
So for example, one of my patients, a woman in her mid-twenties, initially presented to her primary care physician with menorrhagia and fatigue, and her platelet count was found to be 32,000 per microliter. The primary care physician prescribed prednisone 80mg daily and referred her to me. I confirmed the diagnosis of ITP - being on prednisone her platelet count had improved to 123,000 per microliter. Her fatigue, though, was still present and she reported feeling jumpy, so we decided to change her treatment. The patient did not want to

visit the clinic for weekly injections, and she preferred a daily oral treatment without dietary restrictions. So together, we decided to taper her off steroid treatment and start her on Doptelet 20mg daily.

Shawn Streeter:
Thank you for sharing, Doctor Panch. So it sounds like both you and the patient made the decision to start her on treatment with Doptelet, which is a Thrombopoietin receptor agonist, TPO-RA for short. Could you please explain how it works and how it helps raise platelet levels?

Dr Panch:
As we discussed earlier, one of the reasons for the low platelet count typical of ITP is the inhibition of platelet production. Doptelet mimics the action of TPO. TPO is a hormone produced primarily in the liver. Once bound to its receptor, TPO leads to differentiation and proliferation of megakaryocytes, which are the cell type that produce platelets. Doptelet does not compete with TPO for binding to the TPO receptor, and if anything, has an additive effect with TPO on platelet production.

Shawn Streeter:
So we discussed the background of ITP and also individualising the treatment approach for patients. What can you tell us about the study design for the pivotal phase three trial involving Doptelet?

Dr Panch:
So the study included adult patients who had a diagnosis of chronic ITP for more than a year, had received at least one previous therapy for the condition, and had to have two platelet counts, with an average value of the two being less than 30,000 per microliter. The patients were randomised 2 to 1 to daily Doptelet 20mg, which were the 32 patients, or placebo, which included 17 patients. The primary end point of the study was the cumulative number of weeks of platelet response during six months of treatment in the absence of rescue therapy, with platelet count response defined as a platelet count greater than or equal to 50,000 per microliter and the mean baseline platelet count in this study was 14,000 per microliter. So significantly lower.

Shawn Streeter:
Thank you for that overview, Doctor Panch. What parts of the study design inclusion criteria do you find pertinent?

Dr Panch:
As we discussed earlier the clinical definition of chronic ITP is a platelet count of less than 100,000 per microliter over a year, and the study patients had a baseline count of less than 30,000 per microliter so their initial platelet levels were very low. At that level, we would expect to see some of the symptoms mentioned earlier, such as propensity for bleeding, bruising and fatigue, and the study definition of a treatment response was set at a count of at least 50,000 per microliter.

Shawn Streeter:
And then what do the results of the study tell us about the efficacy of Doptelet in managing adult patients with chronic immune thrombocytopenia?

Dr Panch:
So here we can see the results for the primary endpoint.

This graph shows the median platelet count over six months. The onset of effect was seen within the first few days. The median platelet count reached a target range within the first week in fact, before peaking at around two weeks, then remaining in target range for the six month study duration. The median count in the placebo group did not hit the lower bound of the target range at any time. Patients on the Doptelet, obtained platelet count, obtained a platelet count of at least 50,000 per microliter, for a median of 12.4 cumulative weeks, without the need for rescue therapy. The median duration of response in the placebo group was zero weeks. The p value was less than 0.0001, demonstrating a significant difference in these results.

Now the secondary efficacy endpoint was the proportion of patients who responded to treatment by study day eight. Response was defined as reaching a platelet count of 50,000 per microliter, and it was observed that the median platelet count was consistently higher in the Doptelet patient group than the placebo treatment group, starting at day eight, the difference being 80,500 per microliter versus 8000 per microliter in the placebo group. By day eight, 66% of the Doptelet group had reached this target platelet response, but none of the placebo patients did. Over half of those patients that reached the end point, or 37.5% who had reached the end point, had complete response, which was defined as a platelet count of 100,000 per microliter.

Shawn Streeter:
So we have these efficacy results. On the flip side, what does the data suggest about the safety profile of Doptelet?

Dr Panch:

So we have safety data from 128 patients.

And these data are pulled from two phase two, and two phase three trials. There were two discontinuation for adverse events and 12 for serious adverse events. Headache was prominent in the list of adverse events.

In the pooled data the overall exposure to study drug, Doptelet or placebo, was heavily skewed towards Doptelet due to high placebo patient dropout rate for lack of efficacy. So when we look at the treatment engagement adverse events, one may consider adjusting for treatment exposure. In this analysis the adverse event profile was similar between Doptelet and placebo. And so treatment emergent adverse events included headaches headache, fatigue and contusion. So the pooled data revealed no significant Doptelet induced hepatotoxicity as well.

Shawn Streeter:
So moving back to clinical practice again once the patient does start treatment with Doptelet, what is the standard dosing regimen for them and how do you monitor platelet response?

Dr Panch:
So the dosing regimen depends on the patient's response. We use the lowest dose needed for the patient to achieve and maintain a platelet count of at least 50,000 per microliter. The starting dose is a 20mg tablet once daily. There is only one tablet strength, so we adjust the dosage by adjusting the frequency. And this can change from once weekly 20mg tablet all the way to two 20 milligram tablets a day if needed.

The patient I described earlier is a good illustration of how we can adjust frequency of administration based on the platelet count.

If you recall, the patient was initially receiving prednisone and with the introduction of Doptelet, the prednisone was tapered off. And as you see here, the patient's platelet count dropped once the prednisone was ended. So, so we increased her Doptelet dose, and this temporarily resulted in an undesirably high count. But with a couple more titrations we were able to get the count back to a target level.

Shawn Streeter:
And then, are there any other factors that might affect a patient's dosing regimen?

Dr Panch:
Yeah. It was studied whether any dose adjustment was required for East Asian patients, but none was found. Furthermore, Doptelet has no food type restrictions, including calcium. Once we have found the regimen that maintains the patient's platelet level, they can then take Doptelet on a consistent schedule with food.

Shawn Streeter:
So we saw with the short term efficacy in the phase three study, patient population. You've mentioned before that the longer term aim is to maintain a patient's platelet counts. Has long term efficacy been studied with Doptelet?

Dr Panch:
Yes. Patients who completed the six month core phase three study, who also discontinued treatment due to lack of efficacy, were invited to enter an open label extension study. All patients who entered the extension received open label Doptelet, which was titrated with the aim of keeping the platelet count between 50,000 per microliter and 150,000 per microliter. And the mean duration of the extension was 44 weeks, with a range of 8 to 76 weeks. The overall platelet count, or the platelet response rate observed in the core study, was generally maintained throughout the extension phase until around week 36. Over 40% of the study visits found that the patient with a platelet count of at least 50,000 per microliter and this was true in patients who received Doptelet in the core study, and also in those who switched from placebo in the core study to Doptelet in the extension.

Spoken disclaimer:
Further studies are needed to validate the results from these post-hoc analyses. These post hoc analyses may not meet the FDA definition of an adequate and well-controlled study due to their design and inherent limitations. Results from these analyses may differ from those observed in clinical practice. These post hoc analyses are not included in the DOPTELET Prescribing Information.

Shawn Streeter:
Got it. So your example patient previously was able to drop her steroid treatment and maintain platelet count with Doptelet alone. Does response differ between patients?

Dr Panch:
Of course, each patient is different, but the findings of the two post hoc analyses of the core and the extension studies can show evidence in this regard. Over 50% of patients were able to reduce their steroid dose or stop altogether, and this was clinically significant

but not statistically significant.

Shawn Streeter:
And is this response durable?

Dr Panch:
Patient subgroups have been examined based on factors like age or sex, prior therapy, baseline platelet count, and the responses to Doptelet treatment were generally similar across all these subgroups.
Durable response was defined by ASH criteria as platelet count of greater than or equal to 30,000 per microliter, and at least doubling of the baseline count at six months, the mean platelet count increased from 14,000 per microliter to 63,000 per microliter at six months. In addition, another post hoc analysis found that 62% of patients never experienced a loss of response, which was defined as a drop in platelet level below 30,000 per microliter. Patients maintain their initial response, on average for 83.5% of their remaining time in the analysis.

Spoken disclaimer:
Limitations–

1. *This was a retrospective, observational study and was subject to limitations of such a study, such as selection bias and potential confounding, as well as the lack of a defined treatment protocol resulting in heterogeneity of dose adjustment and follow-up frequency.*
2. *There were relatively low patient numbers for each subgroup analysis.*
3. *Non-platelet outcomes were not collected, including bleeding events and health-related quality of life.*
4. *It is not known if patients maintained on romiplostim or eltrombopag would have spontaneously improved their platelet counts had those agents been continued rather than switched to avatrombopag.*

Shawn Streeter:
And we know that Doptelet is not the only TPO-RA used to treat chronic ITP. Do you know of any patients who have switched from one to the other? What were what was their rationale and what was their experience?

Dr Panch:
A retrospective, observational study examined the reasons patients gave for switching from another TPO-RA and their subsequent clinical outcomes. Just over half of the patients who switched did so because of convenience, and this was defined as being able to avoid dietary restrictions and/or the need for weekly injections. Approximately one third switched because their prior treatment lacked efficacy, and the remainder because they had experienced adverse events on their previous therapy. Over 90% of patients who had switched achieved a platelet response or a platelet count of at least 50,000 per microliter. And over 85% achieved a complete platelet response, which was defined as at least 100,000 per microliter.

Shawn Streeter:
Thank you Doctor Panch, for your clinical expertise and for joining us today. Are there any take home messages or points of emphasis that you want to get across to the audience?

Dr Panch:
Thank you. Shawn. I think one of the key points that I would like to make today is to stress upon the importance of the shared decision making process, which has been endorsed by the ASH guidelines as well, both for the patients and for physicians and haematologists. Um, I think it's important to factor in patient's values and preferences while identifying what drug is most optimal for them, and to introduce the pros and cons of each, um, agent that the physician discusses with these patients.

Shawn Streeter:
It was a pleasure having you with us here today. Thank you again.

Dr Panch:
Thank you. Thank you very much.

Announcer:
Doptelet® (avatrombopag) is indicated in adult patients with chronic immune thrombocytopenia who have had an insufficient response to a previous treatment.

Before taking Doptelet, tell your health care provider about all your medical conditions, including if you ever had a blood clot, are pregnant, or plan to become pregnant.

Doptelet may harm your unborn baby. Tell your health care provider if you become pregnant or think you may be pregnant during the treatment with Doptelet. Also tell your doctor if you are breastfeeding or plan to breastfeed.

It is not known if Doptelet passes onto your breast milk. Do not breastfeed during treatment with Doptelet and for at least two weeks after the last dose. Talk to your healthcare provider about the best way to feed your baby during this time.

Tell your health care provider about all the medicines that you take, including prescription and over-the-counter medications, vitamins, herbal supplements. Doptelet may affect the way other medications work, and other medications may also affect the way Doptelet works.

TPO-receptor agonists have been associated with thromboembolic complications in patients with chronic ITP. Thromboembolic events, atrial and venous, have been reported in patients with chronic ITP treated with TPO receptor agonists.

Consider the potential increase in thrombotic risk when administering Doptelet to patients with known risk factors for thromboembolism, including genetic prothrombotic conditions and acquired risk factors.

Monitor platelet counts for signs and symptoms of thromboembolic events and institute treatment promptly. Serious adverse reactions that have occurred more frequently in patients treated with Doptelet compared to placebo was headache.

The most common adverse reactions, occurring in 10% or more of patients with chronic ITP were headache, fatigue, contusion, epistaxis, upper respiratory tract infection, arthralgia, gingival bleeding, petechiae, and nasopharyngitis.

Please see full Prescribing Information at www.Doptelet.com.

References:

1. Lambert MP, Gernsheimer TB. *Blood.* 2017;129(21):2829-2835.
2. Rodeghiero F, Stasi R, Gernsheimer T, et al. *Blood.* 2009;113(11):2386-2393.
3. National Organization for Rare Disorders. Rare Disease Database. Immune thrombocytopenia. Accessed January 2026. https://rarediseases.org/rare-diseases/immune-thrombocytopenia
4. Zufferey A, Kapur R, Semple JW. *J Clin Med.* 2017;6(2):16.
5. Cines DB, Blanchette VS. *N Engl J Med.* 2002;346(13):995-1008.
6. Kistanguri G, McCrae KR. *Hematol Oncol Clin North Am.* 2013;27(3):495-520.
7. Snyder CF, Mathias SD, Cella D, et al. *Curr Med Res Opin.* 2008;24(10):2767-2776.
8. Cooper N, Kruse A, Kruse C, et al.. *Blood.* 2018;132(suppl 1):4804.
9. Neunert C, Terrell DR, Arnold DM, et al. *Blood Adv.* 2019;3(23):3829-3866.
10. Ong LM, de Haes JC, Hoos AM, Lammes FB. *Soc Sci Med.* 1995;40(7):903-918.
11. Committee on Identifying Effective Treatments for Gulf War Veterans' Health Problems. General approach to treating patients. In: Rosof BM, Hernandez LM, eds. *Gulf War Veterans: Treating Symptoms and Syndromes.* Washington, DC: National Academies Press; 2001:43-50. Accessed January 2026.
12. Teutsch C. *Med Clin North Am.* 2003;87(5):1115-1145.
13. Gómez, CA, Kleinman DV, Pronk N, et al. *J Public Health Manag Pract.* 2021;27(6):S249-S257.
14. DOPTELET (avatrombopag) [prescribing information]. Morrisville, NC: AkaRx, Inc; 2025.
15. Kuter DJ. *Blood Rev.* 2022;53:100909.
16. Jurczak W, et al. *Br J Haematol.* 2018;183(3):479-490.
17. Bussel J, et al. Poster presented at: ISTH Conference; July 6-10, 2019; Melbourne, Australia.
18. Al-Samkari H, Aggarwal K, Vredenburg M et al. *Blood.* 2019;134(suppl 1):2356.
19. Jain S, Gernsheimer T, Kolodny S, et al. *Platelets.* 2023;34(1):2195016
20. Nagalla S et al. *Blood* 2019;134(S1):1071.
21. Al-Samkari H, Jiang D, Gernsheimer T, et al. *Br J Haematol.* 2022;197(3):359-366.
22. Data on file. Sobi,Inc.

PP-25541 January 2026