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A Review on Caplacizumab

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Abstract: *Caplacizumab is an advanced targeted therapeutic agent indicated for the management of Acquired Thrombotic Thrombocytopenic Purpura, a rare and potentially fatal hematological disorder characterized by thrombocytopenia, microangiopathic hemolyticanemia, and widespread microvascular thrombosis. This agent is a humanized nanobody that selectively binds to the A1 domain of von Willebrand factor, thereby inhibiting its interaction with platelets and preventing the formation of platelet-rich microthrombi.Caplacizumab has received approval from major regulatory authorities, including the U.S. Food and Drug Administration and the European Medicines Agency, for the treatment of immune-mediated thrombotic thrombocytopenic purpura. It is typically administered in conjunction with plasma exchange and immunosuppressive therapy to facilitate rapid recovery of platelet counts and reduce the risk of disease recurrence.Clinical trials and real-world evidence indicate that Caplacizumab significantly shortens the time to platelet normalization, lowers the incidence of disease exacerbations and refractory cases, and enhances overall survival outcomes. The drug demonstrates a favorable safety profile, with most adverse events being mild, such as epistaxis and headache. Although concerns regarding cost-effectiveness compared to conventional therapy persist, Caplacizumab represents a major therapeutic advancement due to its targeted mechanism and its role in preventing microvascular thrombosis. This review provides a comprehensive overview of its mechanism of action, clinical efficacy, therapeutic applications, and safety profile in the management of acquired thrombotic thrombocytopenic purpura.*

Keywords: Caplacizumab, Cablivi, Acquired Thrombotic Thrombocytopenic Purpura (aTTP), iTTP , ADAMTS13, Nanobody, Plasma Exchange

I. INTRODUCTION

CAPLACIZUMAB
Brand Name: CABLIVI

Your Caplacizumab is a groundbreaking therapeutic agent developed for the treatment of acquired thromboticthrombocytopenic purpura (aTTP), a rare and life-threatening blood disorder characterized by the formation of blood clots in small vessels throughout the body. This drug is a nanobody, a small antibody fragment, whichvspecifically targets and inhibits the activity of von Willebrand factor, a key protein in the clotting process that is overactive in aTTP. By blocking this protein, caplacizumab effectively reduces the formation of dangerous clots, helping to restore normal platelet counts and prevent organ damage. Approved by regulatory agencies such as the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA), caplacizumab represents a significant advancement in the management of aTTP, offering patients a targeted treatment option that addresses the underlying cause of the disorder and improving their overall prognosis. Text (Internet Source) The advent of Caplacizumab, a humanized nanobody that prevents interaction between vonWillebrand factor and the platelet

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glycoprotein receptor.(1) Immune thrombotic thrombocytopenic purpura (iTTP) is characterized by an autoimmune-mediated deficiency, the vWF factor cleaving protease ADAMT 13.(2)iTTP is associated with high morbidity and mortality; if it is left untreated, the mortality rate rise to 90% but falls to 5% to 20% with treatment.(3) Acquired TTP is an acute life-threatening illness characterized by thrombocytopenia and microangiopathic hemolyticanemia (MAHA).(5) Caplacizumab was approved for the treatment of iTTP in 2019.(6) Caplacizumab represents the first drug to receive regulatory approval for the treatment of TTP.(7) FDA approved therapy for rare blood disorder in pediatric patients 12 years and older.(8) This disease is indicated by two primary symptoms: consumptive thrombocytopenia & microangiopathic hemolyticanemia.(9) In 2019, Cablivi became the first FDA approved, nanobody based medicine for acquired (aTTP).(10)TTP is a rare disease with an estimated annual incidence ranging from 1/250,000 to1/1,000,000.(11) Caplacizumab is considered among the advance medications available for the management of iTTP& thrombosis.(12) It works by binding to the A-1 domain of vWF, preventing its interaction with platelets.(13) The murine anti-CD20 monoclonal antibody rituximab being the most successful agent to date to show efficacy in reducing relapse rates.(14) Common Side effects of Caplacizumab also reported by Patients in Clinical trials were bleeding of the nose & headache .(18) Administration of Caplacizumab resulted in a reduced time to platelet count response in an analysis stratified by neurological involvement.(19) The use of Caplacizumab was highly controlled in the clinical trial.(22) Almost one century ago in 1924, TTP was clinically described for the first time by Eli Moschcowitz.(23) The disease is caused by decreased activity of ADAMTS 13.(25) Caplacizumab treatment is generally well-tolerated, hastens platelet recovery, and reduces recurrence rates.(26) Caplacizumab is a humanized single variable domain immunoglobulinfragment that specifically targets the A1 domain of vWF.(27) The phenotypic severity of TTP is affected by multiple epistatic genes and environmental modifiers.(28) aTTP is a rare blood disorder in which blood clots form in small blood vessels.(30) The International Society of Thrombosis &Hemostasis recommends Caplacizumab to be given in combination with PEX + IS (Plasma Exchange + Immunosuppression) for the treatment of an iTTP episode.(31) An episode of TTP may result in long-term consequences, such as cognitive deficits, depression, arterial hypertension, and premature death.(32) The pathophysiology of iTTP involves the inhibition of a Disintegrin and metalloproteinase with thrombospondin motifs 13.(33)

II. PHARMACOLOGY OF DRUG

1. Introduction & Drug Profile

Generic Name: Caplacizumab-yhdp.

Trade Name: Cablivi®.

Therapeutic Class: First-in-class Nanobody® (bivalent humanized single domain antibody fragment).

Indication: Treatment of adult and pediatric patients (12 years and older) with Acquired Thrombotic Thrombocytopenic Purpura (aTTP), in combination with plasma exchange (PEX) and immunosuppressive therapy.

Significance: It is the first FDA-approved therapy (2019) specifically for aTTP, a rare, life-threatening autoimmune blood disorder.

III. HISTORY AND DEVELOPMENT

Caplacizumab was developed as part of a new class of biologic medicines known as nanobodies. Nanobodies are derived from single-domain antibodies originally discovered in camelids. These molecules are much smaller than conventional monoclonal antibodies but retain strong antigen-binding capability. The drug was developed by Ablynx (later acquired by Sanofi) and received approval for treatment of acquired thrombotic thrombocytopenic purpura after successful clinical trials demonstrated its effectiveness in reducing time to platelet count recovery and lowering recurrence of the disease.

2. Drug Classification Pharmacological classification of caplacizumab:

- Antithrombotic agent

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- Monoclonal antibody fragment
- Nanobody (single-domain antibody)
- Anti–von Willebrand factor agent

3. Chemical Nature and Structure

Caplacizumab is a humanized immunoglobulin fragment consisting of a single variable domain antibody. Because of its small molecular size, it can rapidly bind to von Willebrand factor and exert its pharmacological effect.

Key characteristics:

- Recombinant antibody fragment
- High affinity binding to vWF A1 domain
- Rapid onset of action

4. Mechanism of Action

Caplacizumab selectively binds to the A1 domain of von Willebrand factor. This prevents the interaction between vWF and platelet glycoprotein Ib-IX-V receptors.

As a result:

- Platelet adhesion to damaged endothelium is inhibited.
- Platelet aggregation is reduced.
- Formation of platelet-rich microthrombi is prevented.
- Microvascular blood flow improves. This mechanism rapidly reduces thrombosis in patients with aTTP and improves platelet counts.

5. Pharmacodynamics

The pharmacodynamic effects of caplacizumab include inhibition of vWF-mediated platelet adhesion and aggregation. The drug leads to rapid normalization of platelet counts and decreases the risk of thrombotic complications. Clinical studies show that patients receiving caplacizumab experience faster platelet recovery compared to those receiving conventional therapy alone.

6. Pharmacokinetics Absorption:

Caplacizumab is a humanized, bivalent nanobody designed for the treatment of immune thrombotic thrombocytopenic purpura (iTTP). The drug is administered initially as an intravenous bolus dose to ensure rapid onset of action, particularly in the acute phase of the disease. This is followed by daily subcutaneous injections, which provide sustained therapeutic levels and allow for convenient maintenance therapy during the treatment course.

Distribution:

Following administration, Caplacizumab predominantly remains within the intravascular compartment, reflecting its large molecular size and protein-based structure. It circulates extensively in the plasma and demonstrates a high binding affinity for von Willebrand factor (vWF), specifically targeting the A1 domain. By binding to vWF, Caplacizumab effectively inhibits the interaction between vWF multimers and platelet glycoprotein Ib (GPIb) receptors, thereby preventing platelet adhesion and aggregation. This targeted mechanism contributes to its therapeutic efficacy in reducing microthrombus formation.

Metabolism:

Caplacizumab, being a biologic agent composed of protein, is metabolized through standard proteolytic pathways similar to endogenous immunoglobulins. It undergoes enzymatic degradation into smaller peptides and ultimately into amino acids, which are then recycled or utilized in normal metabolic processes. This metabolic pathway does not

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involve hepatic cytochrome P450 enzymes, thereby minimizing the risk of drug–drug interactions commonly associated with small-molecule drugs.

Elimination:
The elimination of Caplacizumab occurs primarily via renal pathways. Both the free (unbound) drug and the drug bound to von Willebrand factor may be cleared from circulation. The pharmacokinetics of Caplacizumab are target-mediated, meaning that its elimination is influenced by the levels of circulating vWF. As a result, the half-life of the drug is not constant but varies depending on the concentration of vWF in plasma. Higher levels of vWF can prolong the drug's presence in circulation due to increased binding, whereas lower levels may result in faster clearance.

7. Therapeutic Indications
Caplacizumab is indicated for the treatment of acquired thrombotic thrombocytopenic purpura (aTTP). It is used in combination with plasma exchange and immunosuppressive therapy. The drug helps prevent formation of new microthrombi while underlying autoimmune causes are treated.

8. Dosage and Administration Typical treatment regimen:
Initial dose: 10 mg intravenous injection before plasma exchange.
Maintenance dose: 10 mg subcutaneous injection daily during plasma exchange therapy.
Continuation therapy: Daily injections for approximately 30 days after completion of plasma exchange.

9. Adverse Effects Common adverse effects:

- Epistaxis (nosebleeds)
- Gingival bleeding
- Injection site reactions
- Headache
- Fatigue Serious adverse effects:
- Severe bleeding complications
- Hypersensitivity reactions Bleeding risk is the most important safety concern because the drug interferes with platelet adhesion.

10. Contraindications and Precautions
Contraindications:

- Hypersensitivity to caplacizumab Precautions:
- Patients with high risk of bleeding
- Patients receiving anticoagulants
- Surgical procedures Patients should be monitored for bleeding symptoms during treatment.

11. Drug Interactions
Potential interactions may occur with drugs that affect blood clotting, including:

- Anticoagulants
- Antiplatelet agents
- Thrombolytic drugs Combined use may increase bleeding risk and requires careful monitoring.

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12. Clinical Trials and Evidence

Clinical trials such as the HERCULES study demonstrated that caplacizumab significantly reduced the time required for platelet count normalization in patients with acquired thrombotic thrombocytopenic purpura. The drug also lowered the incidence of disease recurrence and improved overall clinical outcomes when used with standard therapy.

13. Advantages of Caplacizumab

- Rapid improvement in platelet counts
- Reduced risk of microvascular thrombosis
- Lower recurrence of aTTP
- Targeted mechanism of action

14. Limitations

- Risk of bleeding
- High treatment cost
- Requires daily injections

15. Future Perspectives

Research is ongoing to explore the potential use of caplacizumab in other thrombotic disorders involving von Willebrand factor. Advances in antibody-based therapies may further improve treatment strategies for rare hematologic diseases. (Internet Source)

IV. STUDY BACKGROUND & PURPOSE

The von Willebrand factor directed nanobody Caplacizumab has greatly changed the treatment of immune iTTP in recent years.(2) Data from randomized controlled trials established efficacy & safety. Caplacizumab reduced exacerbation and refractoriness in iTTP.(3) Caplacizumab, an anti-von Willebrand factor humanized bivalent variable domain only immunoglobulin fragment inhibits interaction between von Willebrand factor multimers and platelets.(4) Steroids were given as per local protocol, typically 1 g of methylprednisoloneintravenous daily for the first 3 days of admission.(5) iTTP is a rare hematologic disease caused by autoantibodies against ADAMTS-13 that trigger microangiopathic hemolyticanemia.(6) Given the limited real world data of Caplacizumab, this multicenterrealworld study was designed to assess the safety & efficacy of Caplacizumab in iTTP.(7) TTP is a critical medical condition characterized by thrombocytopenia, hemolyticanemia and organ damage due to microvascular clotting.(9) TTP is a rare & unpredictable disease with a high mortality rate (90%) if untreated.(11) The advanced medication available for the management of TTP is thrombolitik.(12) The addition of Caplacizumab to standard of care treatment to acquired thrombotic thrombocytopenic purpura is not cost effective compared with standard of care alone.(14) Caplacizumab is the only treatment approved by European Agency & the US Food & Drug Administration of iTTP.(15) The microvascular thrombosis caused multiorgan ischemia with potentially life threatening complications.(17) Real world data on Caplacizumab confirm treatment efficiency in 60 patients with aTTP independent of timing & modalities.(19) There is no delay in ADAMTS 13 recovery after PEX starts in Caplacizumab treated patients with iTTP from Spanish registry.(20) The UK real world evidence for Caplacizumab in iTTP represents the largest international data collection outside of clinical trials to date.(22) Initial investigation showed profound thrombocytopenia &schistocytosis on peripheral blood smear.(25) When combined with therapeutic plasma exchange & immunosuppression, upfront universal administration of Caplacizumab was shown to be effective in the management of ITTP.(27) The disorder frequently leads to early death unless the patients are treatedwith plasma exchange or infusion.(28) A recent Phase 2/3 study in Japanese patients showed that Caplacizumab was effective in treating aTTP.(29) In aTTP, an immune-mediated deficiency of the von Willebrand factor cleaving protease.(32)

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V. METHODS & OBJECTIVE

Data Collection:
Demographic, analytical, and clinical data were retrospectively collected from medical records. (1)
Study Objectives:
This study aims to address open questions regarding patient selection, tailoring of therapy duration, and obstacles in prescribing Caplacizumab in iTTP.
Study Design:
The report covers a retrospective, observational cohort of 113 iTTP episodes treated with Caplacizumab versus 119 historical control episodes treated without Caplacizumab.(2)
Access in Spain:
Subjects were identified from the REPTT (Spanish Registry of TTP). During the study period, Caplacizumab was only available in Spain to treat patients with iTTP through the Sanofi Managed Access Program (MAP).(3)
Clinical Trial Reference:
In a separate doubleblind, controlled trial, 145 patients with TTP were randomly assigned to receive Caplacizumab.(4)
Objective:
The study was to determine the safety efficacy & tolerability. Methods: This was a multicentre study in the UK involving the South East England GP study group.(5)
Objective:
The use of Caplacizumab has improved the care of patients with iTTP by reducing time to Platelet Count Normalization, exacerbations, refractoriness, and mortality.(6)
Objective:
TTP management underscores a move towards personalized medicine, focusing on improving outcomes and the quality of life for patients.(8)
Method Overview of models:
These models evaluate the cost-effectiveness of SOC (Standard of Care) plus Caplacizumab versus SOC alone in iTTP based on results.(14)
Objective:
To assess the cost-effectiveness of Caplacizumab in iTTP from the US Payer perspective.(15)
Method:
The National Institute for Health & Care Excellence (NICE) model was adapted to the US setting using US costs and discount rates.(16)
Method (Study Design & Participants):
This study was conducted as a retrospective observational study at 29 German medical centres.(19)
Method:
Caplacizumab marketing authorization for the European Union was released.(20)
Method:
Patients received standard treatment, as per UK national guidance.(22)
Method:
Patients: Recorded clinicobiological data from consecutive adult patients.(23)
Method:
All the patients were treated with daily centrifugal TPE (Therapeutic Plasma Exchange) with plasma and high dose steroids.(27)
Method (Study Population Design & Treatment):
After confirmation of eligibility, patients received 10 mg Caplacizumab once daily.(29)
Method (Statistical Analysis):
A descriptive analysis of the demographic and clinical data was performed.(30)

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Objective & Method: Objective:

To assess the cost-effectiveness of Caplacizumab in iTTP from the US Payer Perspective.

Method (Model Description):

The NICE economic evaluation model, which was developed by researchers at Bresmed Health Solutions, was critically reviewed and iterated upon during the NICE evaluation.(31)

Method (Trial Design & Oversight):

The trial was conducted in collaboration with an external steering committee. An independent data and safety monitoring board monitored the trial heading.(32)

VI. RESULT

Clinical Outcomes and Study Findings

Mortality:

In the early phase of treatment, one patient receiving Caplacizumab and two patients not receiving Caplacizumab died within the first 36 hours of presentation. (1)

Treatment Modalities:

There has been a progressive increase in the use of rituximab as part of combination therapy in the management of thrombotic thrombocytopenic purpura over time. (2)

Clinical Metrics:

Key outcome measures evaluated across studies include time to platelet count normalization, duration of plasma exchange (PEX), and length of hospital stay. (3)

Primary Outcome:

The primary endpoint assessed was the median time to platelet count normalization, analyzed using Kaplan–Meier survival curves and a stratified log-rank test. (4)

Management Approach:

A total of 32 patients received at least four doses of rituximab in accordance with the treatment protocol. (5)

Use of Plasma Exchange:

Plasma exchange therapy was administered in nearly all cases, except for two patients who declined the procedure. (7)

Sensitivity Analysis:

Sensitivity analyses were conducted to determine the factors with the greatest influence on the cost-effectiveness of adding Caplacizumab to standard of care (SOC). (14)

Survival Benefit:

Patients treated with Caplacizumab in combination with plasma exchange and immunosuppressive therapy demonstrated an incremental gain of approximately 2.96 life-years. (15)

Randomized Clinical Trial Findings:

In a randomized cohort of 75 patients, treatment with Caplacizumab significantly reduced the time to clinical response compared to placebo. (17)

Patient Characteristics:

Baseline patient characteristics and initial disease presentations were systematically evaluated to assess treatment outcomes. (19)

Study Population:

A cohort of 108 patients with immune thrombotic thrombocytopenic purpura (iTTP) was analyzed, comprising a total of 113 reported disease episodes during the study period. (20)

Disease Recurrence:

Approximately 6% (5 out of 85) of patients experienced recurrence of thrombotic thrombocytopenic purpura. (22)

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Safety Profile:
Caplacizumab therapy was well tolerated, with no reports of grade 3 adverse events or major bleeding complications. (23)

Participant Characteristics:
Most patients were newly diagnosed cases of TTP, while those presenting with relapse had a median history of three previous episodes. (27)

ADAMTS13 Activity:
A significant reduction in ADAMTS13 inhibitor levels was observed within the first week of treatment initiation in all patients. (28)

Economic Evaluation:
Univariate sensitivity analysis results highlighted key variables influencing cost-effectiveness outcomes. (30)

Efficacy Outcome:
The median time to platelet count normalization was consistently shorter in patients treated with Caplacizumab compared to those receiving placebo. (31)

Systematic Review Findings:
Results from database searches and study selection processes were systematically compiled and analyzed in the review. (33)

VII. DISCUSSION

Overall, these findings are consistent with those reported by Prasannan et al. and should be interpreted in the context of Caplacizumab use in immune thrombotic thrombocytopenic purpura (iTTP).(1) The introduction of Caplacizumab into the therapeutic regimen for iTTP has significantly influenced patient management, adjunctive treatment strategies, and potentially clinical outcomes.(2)

This study provides real-world evidence regarding the efficacy and safety of Caplacizumab in patients with iTTP managed in Spain.(3) Notably, disease exacerbations were observed up to 25 days after cessation of plasma exchange (PEX), supporting the continued use of Caplacizumab during the high-risk period.(4) The effectiveness of PEX is well established; however, patients with anti-ADAMTS13 antibodies often require more intensive PEX and increased plasma utilization.(5)

Importantly, patients treated with Caplacizumab did not experience major hemorrhagic events or significant complications.(7,26) Autopsy findings in affected patients have demonstrated widespread microvascular thrombosis across multiple organs.(9) Caplacizumab plays a critical role in controlling platelet aggregation until immunosuppressive therapies, which have a delayed onset of action, become effective.(11)

In the TITAN trial, the use of Rituximab was more frequent among patients receiving Caplacizumab compared to those receiving standard of care (SOC).(14) Caplacizumab inhibits microthrombus formation, thereby reducing the risk of exacerbation, refractory disease, and mortality during the acute phase.(15) This is further supported by faster platelet count normalization, indicating reduced platelet consumption and prevention of progressive tissue ischemia.(17)

The study population predominantly consisted of patients of white descent, with most cases presenting as a first episode of acquired TTP rather than relapsing disease.(19) The restoration of ADAMTS13 activity should not be a limiting concern when considering Caplacizumabtherapy.(20)

This represents one of the largest international collections of real-world data evaluating Caplacizumab outside formal clinical trials.(22) Additionally, guidelines from the International Society on Thrombosis and Haemostasis (ISTH) have introduced risk assessment scores to predict ADAMTS13 activity with high diagnostic accuracy.(23)

Autoimmune disorders, particularly systemic lupus erythematosus (SLE), are among the leading causes of acquired TTP and are associated with higher mortality rates and poorer treatment responses.(25) Integrated analyses from the TITAN and HERCULES trials demonstrated a median time to platelet normalization of 2.75 days with early initiation of Caplacizumab.(27)

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Furthermore, ADAMTS13 activity and inhibitor levels were found to correlate with patient clinical status during treatment.(29) A post hoc integrated analysis of the HERCULES and TITAN trials also evaluated survival outcomes, healthcare utilization, organ damage, and refractory iTTP.(30)

Given that iTTP is an ultra-rare but life-threatening condition with significant long-term consequences if inadequately treated, early and effective intervention is critical.(31) Relapses were observed predominantly in patients with persistently suppressed ADAMTS13 activity below 10%.(32) Therefore, one of the key clinical factors influencing the efficacy of Caplacizumab is the timing of its initiation, with earlier use associated with improved outcomes.(33)

VIII. CONCLUSION

Caplacizumab, marketed as Cablivi, represents a significant advancement in the treatment of acquired Thrombotic Thrombocytopenic Purpura (aTTP), a rare and life-threatening blood disorder. It is the first drug approved by the FDA & EMA for the treatment of aTTP. As a humanized nanobody, Caplacizumab specifically targets the A1 domain of von Willebrand factor (vWF) preventing its interaction with platelets & reducing the formation of microthrombi. This mechanism helps restore platelet count, prevents organ damage & reduces recurrence rates. Clinical trials &real world studies have demonstrated its efficacy in reducing time to platelet count normalization, exacerbation, refractoriness & mortality when combined with plasma exchange (PEX) & immunosuppressive treatments. Caplacizumab has shown to be effective in managing aTTP episodes. It is generally well-tolerated without common side effects including nosebleeds & headache and no major haemorrhagic events reported in studies. Despite its benefits, the addition of Caplacizumab to standard care is not considered cost effective compared to standard care alone. However, its ability to prevent platelet consumption, tissue ischemia & progression of the disease makes it a valuable option for improving patient outcomes in the acute phase of aTTP.

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