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Case Report

Long-Acting Sandostatin as an Effective Therapy for Osler-Weber-Rendu Disease: A Case Report

Rim Boutari*[1], Mahmoud Hallal[1], Antoine Abou Rached[1]
[1]Lebanese University, Faculty of Medical Sciences, Beirut, Lebanon

***Corresponding author:** Rim A. Boutari, Lebanese University, Faculty of Medical Sciences, Beirut, Lebanon

Abstract

Background: Hereditary Hemorrhagic Telangiectasia (HHT), also known as Osler-Weber-Rendu disease, is a genetic disease that follows an autosomal dominant inheritance pattern. It is characterized by the presence of mucocutaneous telangiectasias, which are small dilated blood vessels found on the skin and mucous membranes. Individuals with HHT most commonly present with epistaxis and gastrointestinal bleeding, that can subsequently lead to the development of iron deficiency anemia.

Case report: Here we describe the case of a 54-year-old female patient with a known history of diabetes mellitus and HHT. She developed severe iron deficiency anemia secondary to gastrointestinal bleeding, necessitating intravenous iron replacement and multiple blood transfusions.

The patient was put on long-acting Sandostatin, at a dose of 20mg given intramuscularly once per month. Following the initiation of Sandostatin, the patient experienced a resolution of melena and stabilization of hemoglobin levels.

Conclusion: This case demonstrates the potential effectiveness of long-acting Sandostatin in the treatment of Osler-Weber-Rendu disease.

Keywords: Osler-Weber-Rendu, telangiectasias, gastrointestinal bleeding, Sandostatin, case-report.

Introduction

Osler-Weber-Rendu disease also called Hereditary Hemorrhagic Telangiectasia (HHT) is an uncommon genetic vascular disease, of autosomal dominant inheritance, that is mainly caused by ACVRL1 (activin receptor-like kinase 1), ENG (endoglin), and MADH4/SMAD4 heterozygous gene mutations [1,2].

These mutations lead to various vascular abnormalities including small telangiectasias and larger arteriovenous malformations. Mucocutaneous telangiectasias, the dilation of postcapillary venules directly connected to dilated arterioles, is a key feature which can occur in different organs such as the gastrointestinal tract, nose, liver, lungs, brain and occasionally the pancreas [2,3]. These dilated micro vessels can easily bleed because their walls are fragile and blood flow is turbulent[3].

Patients with HHT often present with recurrent epistaxis, gastrointestinal bleeding, and various systemic manifestations, including iron deficiency anemia [2,3].

The management of HHT is primarily focused on symptomatic relief and prevention of complications [4]. Treatment approaches for HHT are constantly evolving, especially with the emergence of antiangiogenic therapies. These therapies aim to address bleeding telangiectasias and achieve hemostasis[5].

The latest clinical guidelines for diagnosing and treating HHT now include systemic therapies such as antifibrinolytics

and antiangiogenics as standard treatment choices for severe HHT-related gastrointestinal bleeding[6].

The use of Sandostatin, a long-acting somatostatin analog, has shown promising results in certain cases [7].

Herein, we present a patient with HHT and iron deficiency anemia who demonstrated a positive response to long-acting Sandostatin therapy.

Case Presentation

In this case, a 54-year-old woman with a preexisting condition of diabetes mellitus and HHT, came in with recurring episodes of melena and related iron deficiency anemia. Physical examination revealed characteristic telangiectasias on the tongue and lips, which further supported the diagnosis of HHT (figure 1).

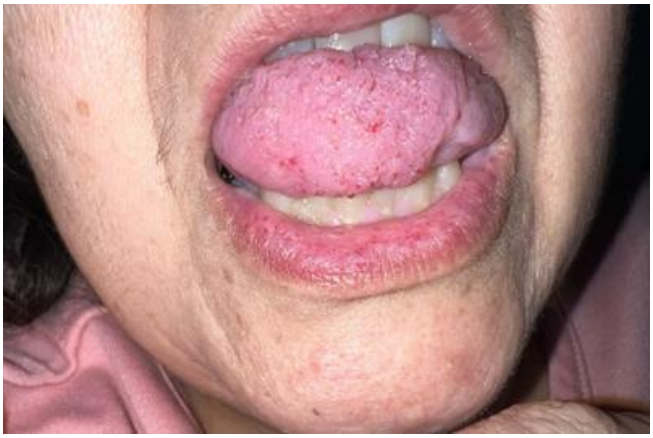

Figure1: Characteristic telangiectasias on lips and tongue

The patient underwent esophagogastroduodenoscopythat revealed the presence of multiple duodenal angiodysplasias (figure 2).

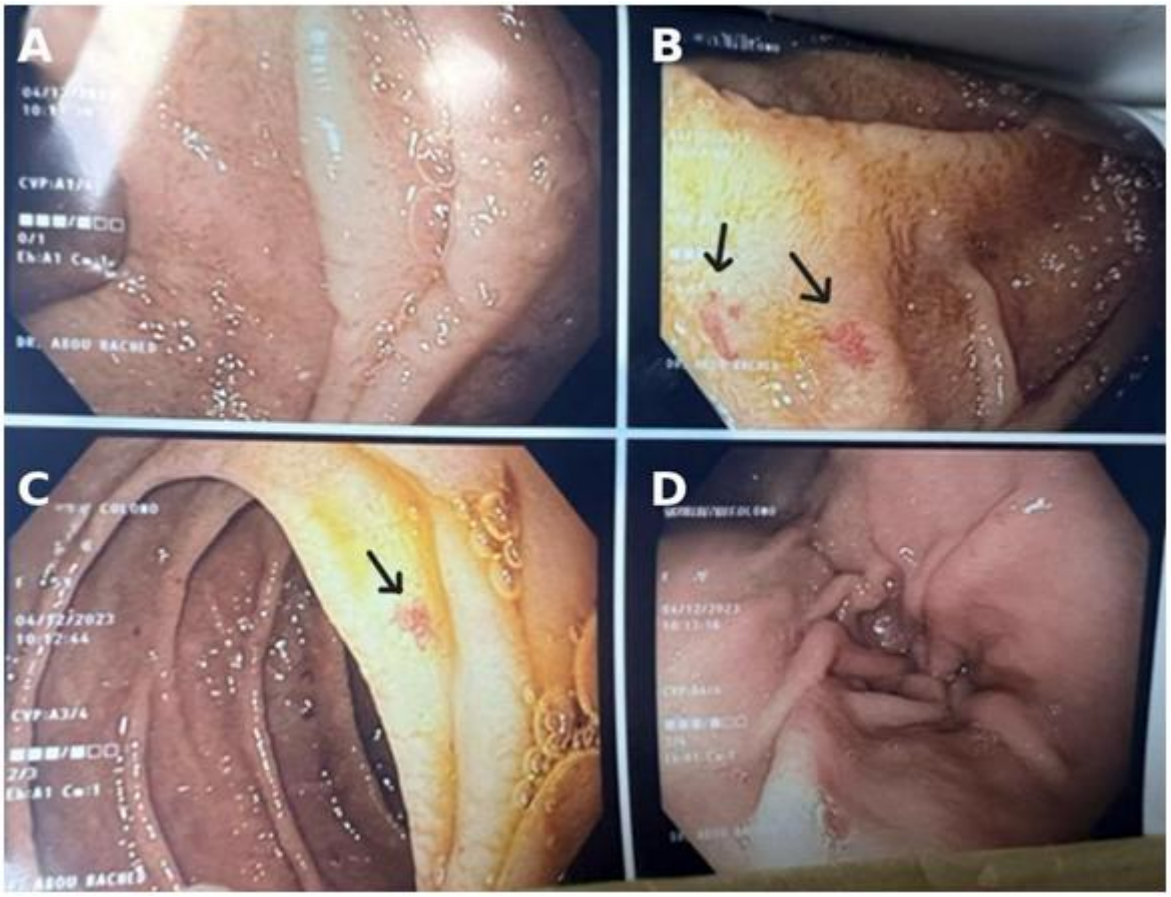

Figure 2: EGD showing duodenal angiodysplasias

She had previously required intravenous iron replacement and multiple blood transfusions to manage her recurrent severe anemia.

In light of the persistent bleeding and anemia, long-acting Sandostatin therapy was initiated at a dose of 20mg intramuscularly once per month.

Following the initiation of Sandostatin, the patient experienced a remarkable improvement in her clinical condition. The episodes of melena ceased, and subsequent laboratory investigations revealed stabilization of her hemoglobin levels (Table 1).

Table 1: Patient lab values before and after initiation of sandostatin

Time period	Hgb (g/dL)
3 months prior to sandostatin	7.6 g/dL
2 months prior to sandostatin	8.2 g/dL
1 month prior to sandostatin	7.4 g/dL
1 month after sandostatin	10.3 g/dL
2 months after sandostatin	11.2 g/dL
6 months after sandostatin	11.6 g/dL

The patient mentioned experiencing a substantial improvement in her quality of life, along with a decrease in fatigue and an overall increase in well-being.

No adverse effects related to Sandostatin therapy were reported during the course of treatment.

Discussion

Hereditary Hemorrhagic Telangiectasia, Or HHT, is a genetic disease that manifests by vascular lesions such as arteriovenous malformations and mucocutaneous telangiectasias, which can appear in various locations throughout the body including the gastrointestinal tract, liver, spleen, central nervous system, fingers and nasopharynx[8]. These lesions have a high propensity for bleeding resulting in, most commonly, spontaneous and recurrent epistaxis or gastrointestinal bleeds with subsequent chronic iron deficiency anemia[7,8]. The prognosis of patients with HHT depends on the seriousness of their symptoms. Generally, the prognosis is positive as long as bleeding is quickly detected and effectively managed [9].

The Curaçao criteria, established in June 1999, are used to clinically diagnose HHT [4].These criteria take into consideration the presence of epistaxis, telangiectasias, visceral lesions and family history. If a person meets at least three of these criteria, the diagnosis is said to be definite. On the other hand, if two criteria are present, it is classified as suspected or possible. [4,5]. While if less than two criteria are present, the diagnosis is considered unlikely. HHT has a variable penetrance and expression so even family members having the same genetic mutation can present different symptoms of varying severity [10]. That may explain the fact that approximately 20% of patients with HHT may not even realize that they have a positive family history [11].

Concerning gastrointestinal bleeding, the expert panel suggests grading its severity as follows: mild if patient's anemia can be managed by oral iron supplements, moderate if patients need intravenous iron replacement to maintain their hemoglobin level, and severe if patients are unable to reach their hemoglobin goals even with iron replacement or in patients who require blood transfusions to do so [6].

Medical treatment, as per the latest guidelines, include the use of antifibrinolytics like tranexamic acid for mild gastrointestinal bleeding related to HHT, and the use of systemic antiangiogenics or intravenous bevacizumab for moderate or severe bleeding related to HHT [6].

The goal of treatment in HHT is to decrease the frequency and severity of bleeding episodes in order to maintain hemoglobin levels, control anemia and improve patient's quality of life, since most patients having HHT suffer from discomfort, anxiety and depression [11,13].

Somatostatin analogs (SA) have been utilized for quite some time in patients without HHT having intestinal angiodysplasias and a new study proved its efficacy in HHT patients as well[7,12]. In this particular study, researchers included a group of 119 patients who had HHT with gastrointestinal involvement. Out of these patients, 67 received somatostatin analogs as treatment. The median duration of treatment was 20.9 months, with a standard deviation of 15.2 months. Notably, the patients treated with somatostatin analogs demonstrated significant improvement in hemoglobin levels and a reduced requirement for red blood cell transfusions [7].

Back to our case, our patient was having severe HHT-related gastrointestinal bleeding that required to start her either on intravenous bevacizumab or other antiangiogenic therapy. Since bevacizumab is quite expensive and of limited availability, we opted to begin her treatment with a monthly intramuscular dose of 20mg of long-acting Sandostatin, a somatostatin analog.

After starting her on long-acting Sandostatin, the patient didn't experience any episode of melena, and her hemoglobin level remained stable for a period of 6 months since starting the therapy. She also noticed an improvement in her quality of life and a reduction in fatigue. Moreover, she hasn't reported any side effects while taking the treatment.

This indicates that somatostatin analogs are effective in treating gastrointestinal bleeding related to HHT.

Conclusion

The management of HHT is continuously advancing especially with the emergence of antiangiogenic therapies. This article, highlights the positive impact of antiangiogenic treatments, specifically long acting Sandostatin, in managing HHT. In this case, the initiation of long-acting Sandostatin in a 54-year-old female patient known to have HHT and diabetes mellitus resulted in the ending of melena and stabilization of hemoglobin levels. These findings suggest that Sandostatin can be an effective therapy for managing bleeding associated with Osler-Weber-Rendu disease. Further studies and clinical trials are warranted to explore the full potential of Sandostatin in the treatment of HHT and to validate our findings.

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