

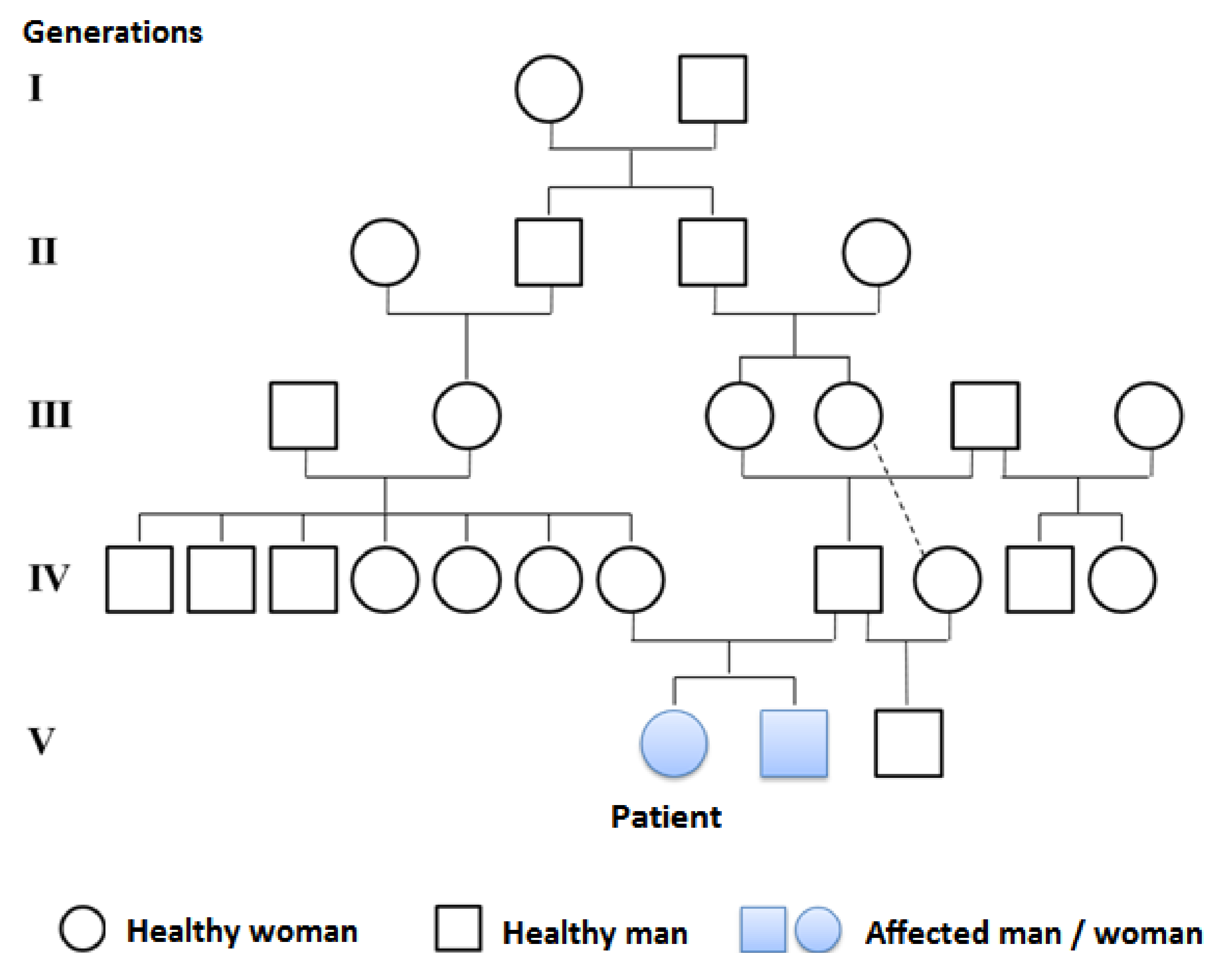
SUCCESSFUL OFF-LABEL USE OF ELTROMPOBAG IN A PREGNANT WOMAN WITH CONGENITAL THROMBOPENIA

J. Raymond¹, J. Descout¹, A. Aouba², S. Barbault-Foucher¹, A. Rieutord¹, A. Decottignies¹.

¹Hôpital Antoine Bécère- APHP, Pharmacie, Clamart, France. ²Hôpital Antoine Bécère- APHP, Médecine Interne, Clamart, France.

Background

Congenital thrombopenia are rare diseases. We report the case of a **pregnant 25-year-old woman**, suffering from a new form of **severe congenital thrombopenia** due to an autosomal recessive **mutation in PRKACG gene**, found in 2014^a. This mutation is responsible for **platelet release deficiency**, revealed in childhood by oral bleedings, epistaxis and a platelet count around 10 G/L. She received repeated platelet transfusions, leading to isoimmunisation and resistance to treatment. Her brother is also affected by this disease, in a **consanguineous family context** (see opposite). The patient was advised not to plan such a **high-risk pregnancy**. A medical termination of pregnancy, proposed at 18 weeks of amenorrhea (WA), was rejected by the couple.



Purpose

The aim of this work was to report the off-label use of eltrombopag in a pregnant woman with congenital thrombopenia, allowing reaching a suitable platelet count for a **safe delivery**, scheduled at 35-37 WA: 30-50 G/L for a vaginal birth and at least 50 G/L for a caesarean.

Conclusion

Although eltrombopag is **contraindicated** during pregnancy, due to lack of data, its **off-label use** was the **most effective treatment** in the management of this patient's thrombopenia and it enabled a high-risk pregnancy to achieve a successful outcome.

Materials and Methods

Several **off-label drugs** were successively tested:

Immunoglobulin G - 1 g/kg/day - Intravenous route

↳ **THROMBOPOIETIN RECEPTOR AGONISTS:**

Romiplostim - 250 µg / week - Subcutaneous route

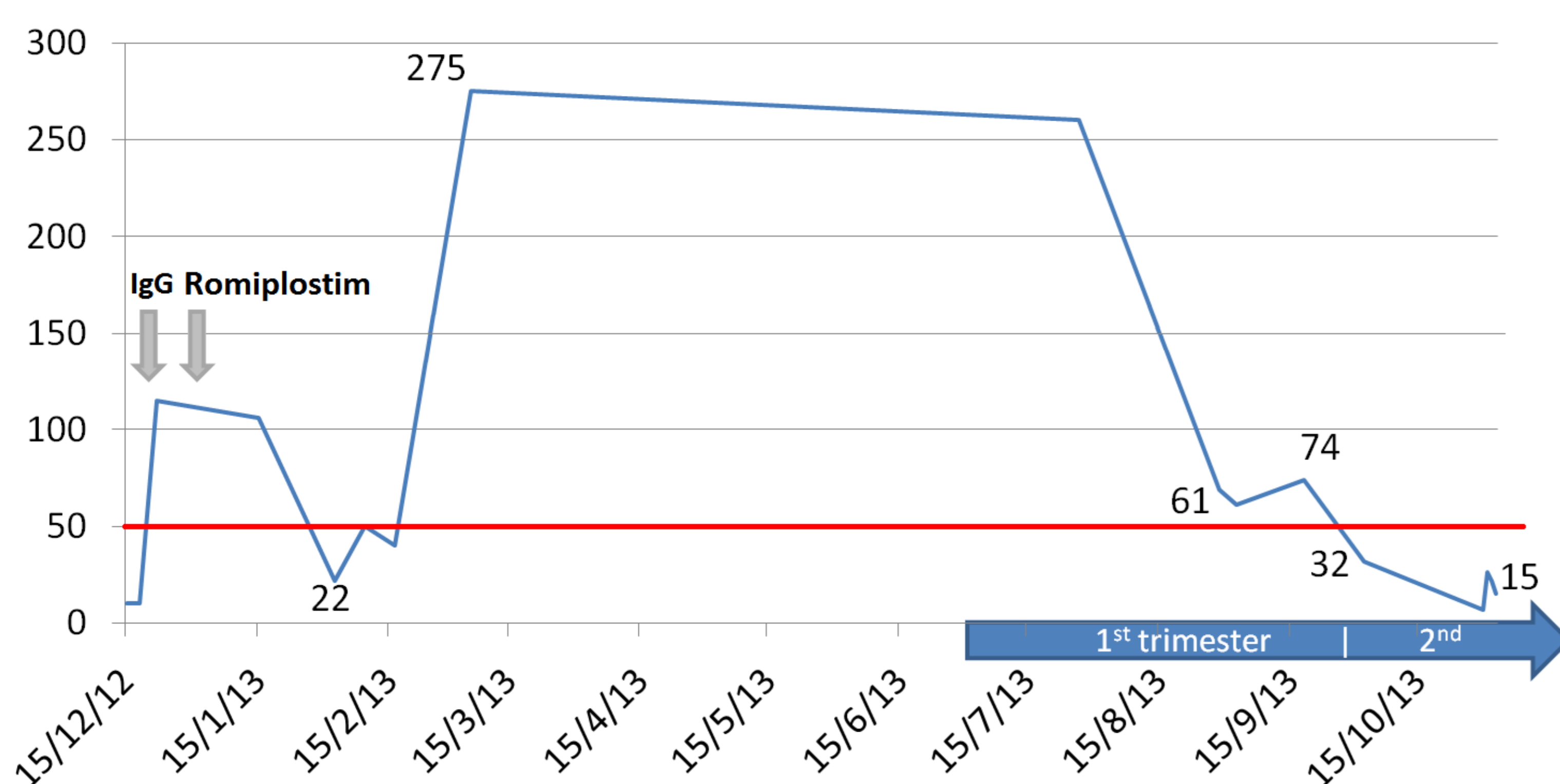
↳ **Eltrombopag** - 50 to 125 mg / day - Oral route

A **multidisciplinary committee** including hematologists, internists, obstetricians, pharmacists and pharmacologists from the Center for Teratogenic Agents took the decision based on a risk-benefit approach.

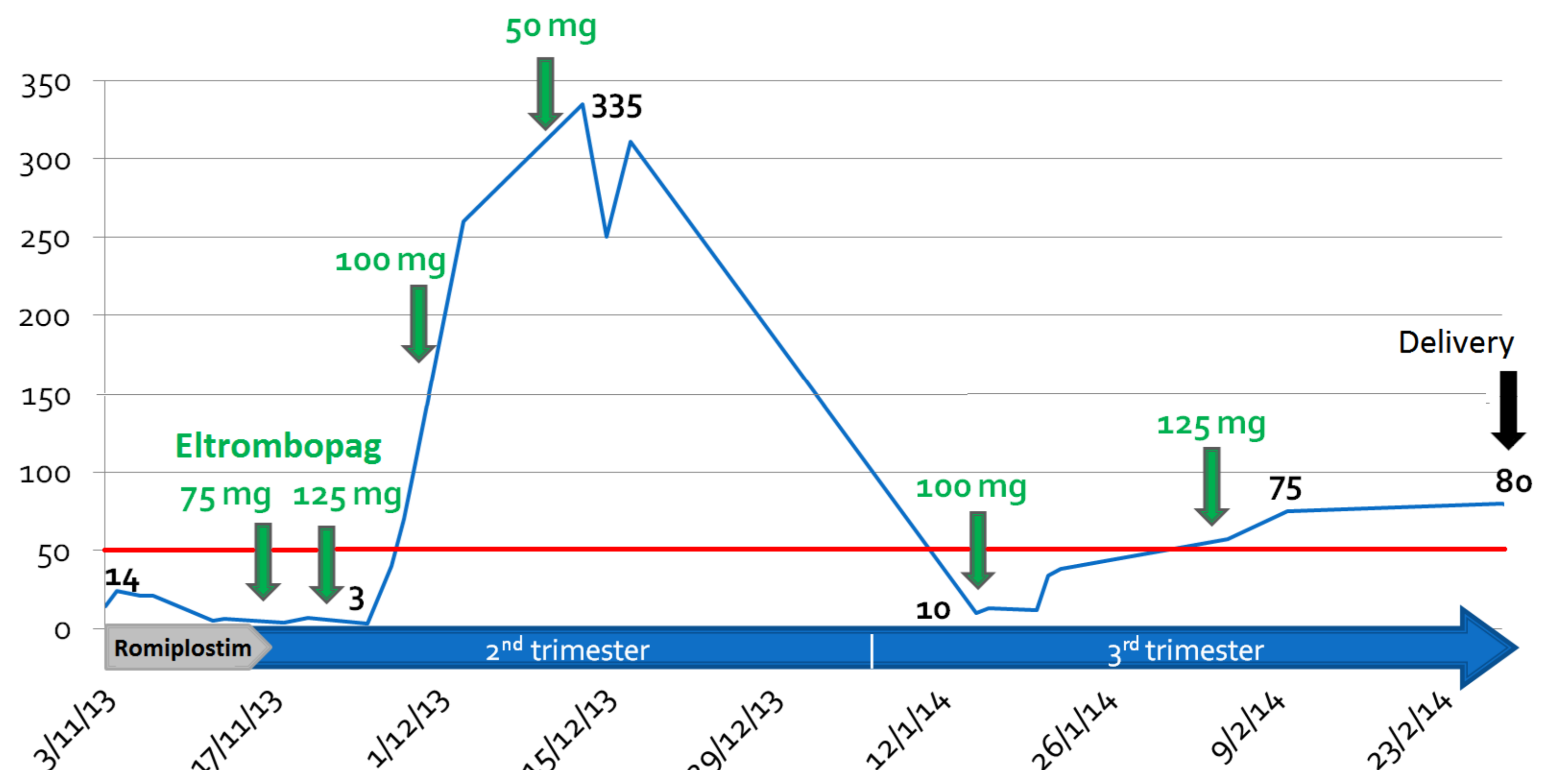
Twice-monthly **platelet counts** were performed and the dose of eltrombopag was adjusted accordingly. **Ultrasound** was monthly performed for foetal monitoring.

Results

Platelet count (G/L) during immunoglobulin G (IgG) and romiplostim treatments



Platelet count (G/L) during eltrombopag treatment, and dose adjustments



The patient gave birth by caesarian at 35 WA and 2 days, without hemorrhagic complication and her platelet count was 80 G/L. The newborn was in good health and his platelet count was 250 G/L. He is not affected by the disease.

^aA new form of macrothrombocytopenia induced by a germline mutation in PRKACG gene. *Blood*. 2014 Jul 24. Manchev VT, Hilpert M, Berrou E, Elaib Z, Aouba A, Boukour S, Souquere S, Pierron G, Rameau P, Andrews R, Lanza F, Bobe R, Vainchenker W, Rosa JP, Bryckaert M, Debili N, Favier R, Raslova H.