PO239

Management of Glanzmann Thrombasthenia: A European Survey On Current Clinical Practice

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BACKGROUND & AIMS

- Glanzmann thrombasthenia (GT) is a rare and complex bleeding disorder, of which diagnosis and management is particularly challenging.
- Expert opinions and registry data have informed some existing guidelines and recommendations, but there remains a lack of consensus on optimal approaches in clinical practice.
- This initiative, the first of the EAHAD GT Working Group, aimed to explore current European clinical practice via a physician survey.
- Additional information on the background and formation of the EAHAD GT Working Group can be found in Poster PO278.

MATERIAL & METHODS

- European haematologists were invited to an online 57-question questionnaire, covering various aspects of GT diagnosis and management.
- Survey topics included: laboratory testing; platelet and RBC administration; anti-HLA and anti-αIIbβ3 immunisation; use of rFVIIa; and other treatments.
- Questions were either single or multiple-choice, with text boxes for qualitative responses.
- The survey was administered via SurveyMonkey (www.surkeymonkey.com) and launched publicly at the 2023 EAHAD congress in the United Kingdom.
- Data collected between 23 January and 16 June 2023 were pooled before analysis to ensure respondent anonymity and tallied using simple descriptive statistics.

RESULTS: SURVEY RESPONDENTS

- 92 complete responses from European countries of interest were included for analysis (Figure 1).
- Plus, Sudan (n=1) and Turkey (n=1), both from centres with >30 patients.

Figure 1 Geographic distribution of responses (n=92) from countries included in analysis

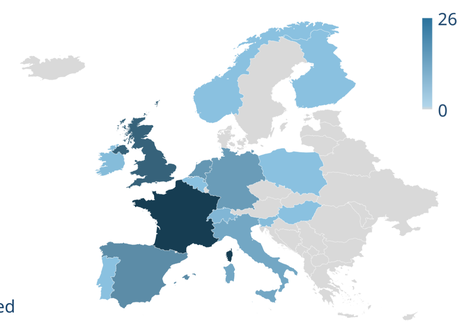
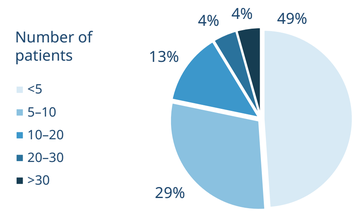


Figure 2 Responses by size of centre, arranged by number of patients followed-up (n=92)



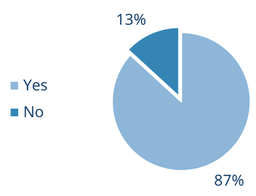
- On average, most responses came from centres that follow up <10 patients (Figure 2), illustrating the rarity of GT in the European population.

RESULTS: SURVEY OUTCOMES

Similarities in clinical management approaches

Phenotypic characterisation

Figure 3 Proportions of respondents who screen patients with GT for HLA antibodies (n=91)



- Almost all respondents perform light transmission aggregometry, flow cytometry and genetic analysis to diagnose patients with GT.
- About 90% of respondents screened for anti-HLA antibodies (Figure 3).

HLA-matched platelets

- Almost three quarters of respondents would use HLA-identical apheresis platelet concentrates if available.

Dosing of rFVIIa

- 87% of respondents used the recommended dose of rFVIIa (80–120 µg/kg body weight every 2–3 hours).

Haemostatic cover in minor surgical settings

- Around 80% of respondents would administer 1 unit of platelets for minor surgery (Figure 4).
- When asked how many days of rFVIIa they would propose in cases of minor surgery, over 80% of respondents reported fewer than 2 days (Figure 5).

Figure 4 Units of platelets administered by respondents in cases of minor surgery (n=81)

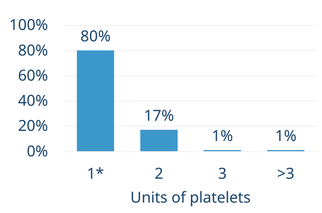
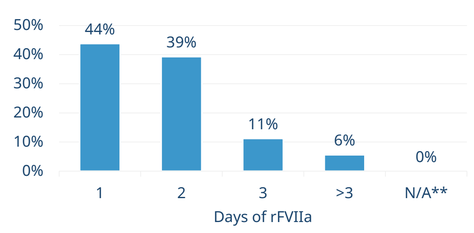


Figure 5 Days of rFVIIa administered in cases of minor surgery (n=89)



Other treatment

- Three quarters of participants prescribed chronic oral iron supplementation to their patients.

Differences in clinical management approaches

Circumstances of HLA-matched platelet use

- Only 32% of respondents used HLA-matched platelets systematically (Figure 6). The most common indication in which other respondents might use HLA-matched platelets was in cases of historical refractoriness without current immunisation (Figure 7).

Figure 6 Proportions of respondents who use HLA-matched platelets systematically versus in specific indications for treatment of patients with GT (n=91)

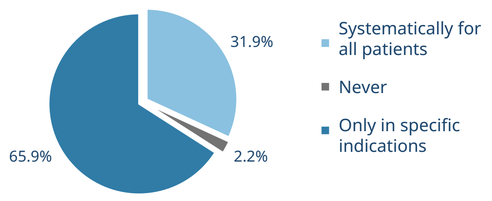
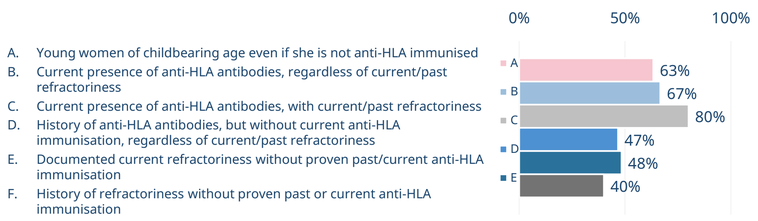


Figure 7 Indication-specific use of HLA-matched platelets in treatment of patients with GT (n=60)



Haemostatic cover in major surgical settings

- Respondents used different amounts of haemostatic cover in major surgical settings (Figures 8, 9, 10).
- Almost two thirds did not use pharmacological thromboprophylaxis in surgical settings.

Figure 8 Units of platelets administered by respondents in cases of major surgery (n=86)

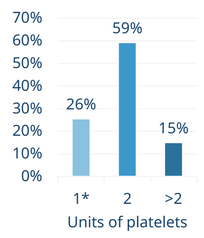


Figure 9 Days of platelets administered by respondents in cases of major surgery (n=84)

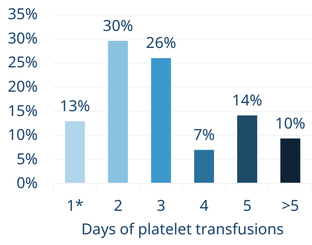
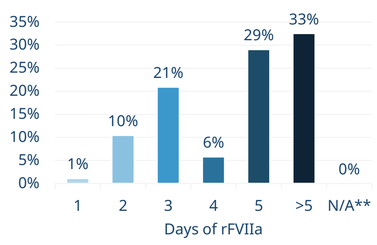


Figure 10 Days of rFVIIa administered by respondents in cases of major surgery (n=86)



Consideration and screening of αIIbβ3 antibodies

- Although over 80% of respondents reported that anti-αIIbβ3 antibodies at least sometimes represent a clinical concern, fewer than half regularly screen for anti-αIIbβ3 antibodies (Figures 11, 12).

Figure 11 Proportions of respondents who consider αIIbβ3 antibodies a clinical concern in management of GT (n=90)

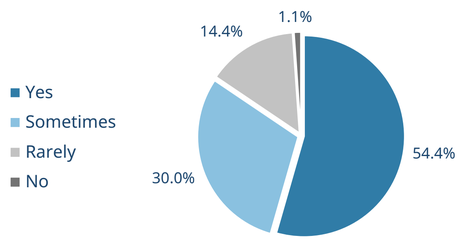
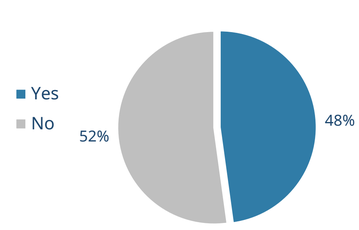


Figure 12 Proportions of respondents who screen for anti-αIIbβ3 antibodies in patients with GT (n=92)



CONCLUSIONS

- Variation in practice could reflect the lack of evidence-based recommendations in the management of GT. International consensus and guidance may facilitate better care and improved outcomes for people living with GT and its manifestations.
- This survey represents a prioritisation of the most clinically relevant topics for practicing haematologists, as selected by the author group. Further investigation could explore additional topics in disease management, such as pregnancy and/or additional therapies, and may be covered in subsequent surveys.

Footnotes and abbreviations:
**1 day of therapy administration includes pre-surgical administration*
*** N/A, not applicable, would not use*

αIIbβ3, integrin αIIbβ3; GT, Glanzmann Thrombasthenia; HLA, human leukocyte antigens; n, number of respondents; RBC, red blood cell; rFVIIa, recombinant activated factor VII

ACKNOWLEDGEMENTS

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DISCLOSURES

M.F. has received speaker's fees from CSL Behring, Novartis, Novo Nordisk, Sobi, LFB, BMS and Sanofi. **R.E.S.** has received speaker's fees from Bayer, CSL Behring, Hemab, Novartis, Novo Nordisk, Octapharma, Roche, Sobi and Takeda. **M.M.** has received speaker's fees from Octapharma, Novo Nordisk, Sanofi, Roche and Sobi. **A.A.** has received speaker's fees from Sanofi. **R.K.** has received speaker's fees from Bayer, BioMarin, Biotest, BMS, CSL Behring, Daiichi Sankyo, Grifols, LEO, Novo Nordisk, Octapharma, Pfizer, Roche, Sanofi, Sobi and Takeda. **R. D'O.** has received speaker's fees from Takeda, BioMarin, CSL Behring, LFB, NovoNordisk, Octapharma, Roche/Chugai, Sobi/Sanofi, uniQure and Spark.