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Aplastic Anemia (short version)

Recommendations from the society for diagnosis and therapy of haematological and oncological diseases

DGHO DEUTSCHE GESELLSCHAFT FÜR HÄMATOLOGIE UND MEDIZINISCHE ONKOLOGIE

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Medizinische Onkologie e.V.
Bauhofstr. 12
D-10117 Berlin

Executive chairman: Prof. Dr. med. Andreas Hochhaus

Phone: +49 (0)30 27 87 60 89 - 0

info@dgho.de
www.dgho.de

Contact person

Prof. Dr. med. Bernhard Wörmann
Medical superintendent

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Aplastic Anemia (short version)

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Compliance rules:

- Guideline
- Conflict of interests

Authors: Hubert Schrezenmeier, Tim Henrik Brümmendorf, Hans Joachim Deeg, Britta Höchsmann, Sigrid Machherndl-Spandl, Jens Panse, Jakob Passweg, Alexander Röth, Jörg Schubert, Bernhard Wörmann

1 Summary

Aplastic anemia (AA) (synonyms: panmyelopathy, panmyelophthisis) comprises a heterogeneous group of rare diseases leading to bone marrow insufficiency. The more common acquired aplastic anemias are to be distinguished from the 'inherited bone marrow failure syndromes'. Clinically, aplastic anemia is dominated by the symptoms of bi- or tricytopenia with anemia, neutropenia, thrombocytopenia in various combinations and to variable degrees.

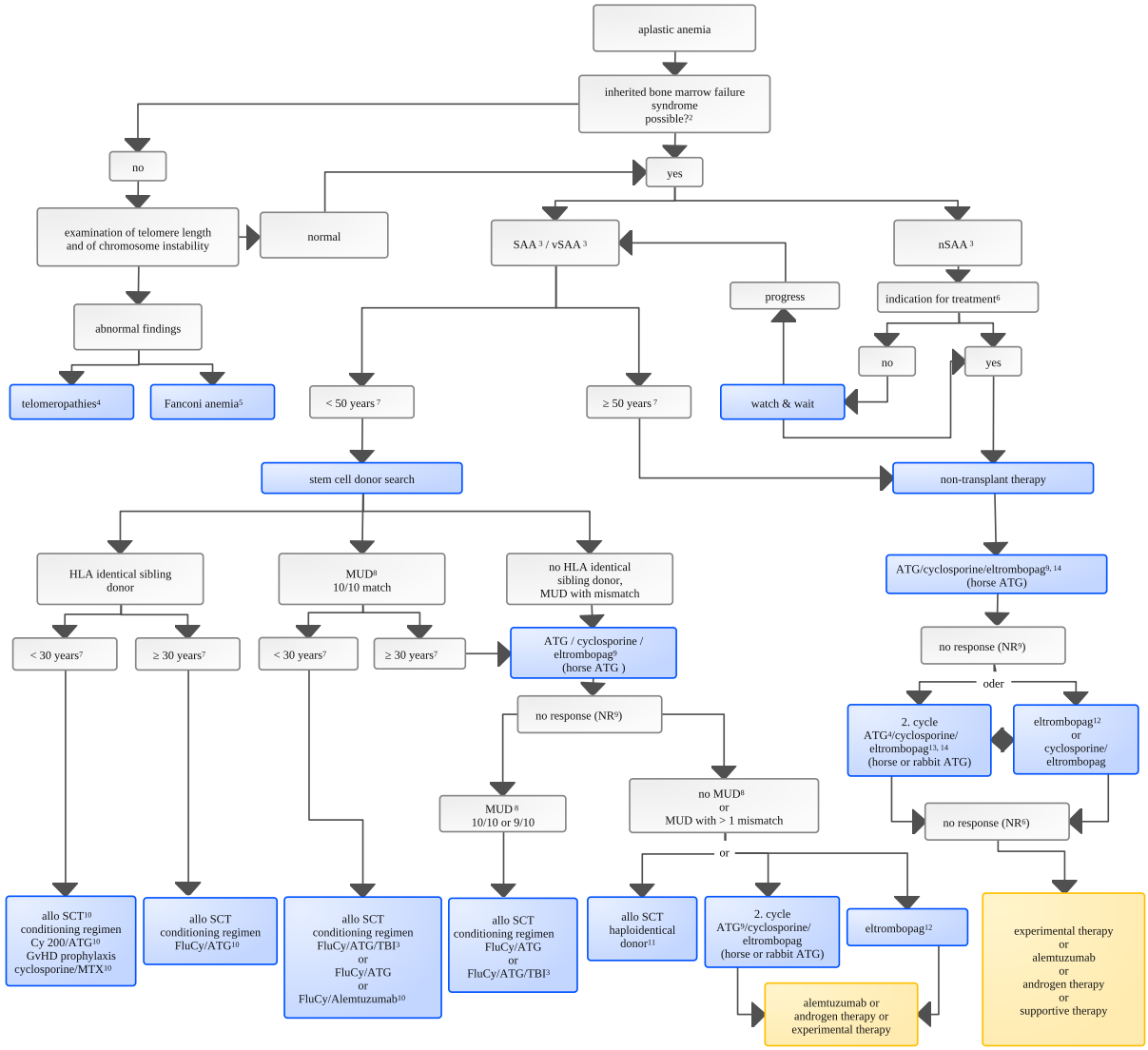
Therapy is based on the etiology and especially on the clinical manifestation. In cases of non-severe/moderate aplastic anemia (nSAA/MAA), wait-and-see approach is recommended or, if treatment is indicated, immunosuppressive therapy. In severe or very severe aplastic anemia, allogeneic stem cell transplantation is curative. If not feasible, immunosuppressive therapy with horse anti-thymocyte globulin (ATG) plus ciclosporin A (CSA) is the gold standard in first-line treatment in these cases. New drugs such as the thrombopoietin receptor eltrombopag expand the therapeutic options.

As aplastic anemia is a rare disease and often requires further therapy optimization. Contact should be made with an expert center before initiating therapy to clarify, among other things, whether treatment is possible in the context of a clinical trial.

2 Therapy

The current treatment algorithm is depicted in Figure 1.

Figure 1: Therapy algorithm for aplastic anemia



Legend:
curative treatment intent; non-curative treatment intent;
blue: established therapy, yellow: experimental therapy

[1] recommendations for children in protocols and guidelines of the Pediatric Study Group Aplastic Anemia;

[2] see chapter 6. 3 (Link to German guideline)

[3] nSAA - non-severe AA (aplastic anemia); SAA – severe AA, vSAA - very severe AA

[4] see chapter 6. 3. 1 (Link to German guideline)

[5] see chapter 6. 3. 2 (Link to German guideline)

[6] see chapter 6. 1. 1 (Link to German guideline)

[7] important is the biological age and not the age in years; this is important for patients ≥50 years, who do not respond to non-transplant therapy

[8] MUD – matched unrelated donor; see chapter 6.1.3.2.1 (Link to German guideline)

[9] first line standard therapy for patients with vSAA / SAA, who are ineligible for allogeneic stem cell transplantation is the combination of ATG
(anti-thymocyte globulin), ciclosporine A and eltrombopag. (Eltrombopag is not approved for this indication in Germany).

[10] allo SCT: allogeneic stem cell transplantation; Cy200 - cyclophosphamide 200 mg/kg, FluCy/ATG – fludarabine, low dose cyclophosphamide and ATG; MTX – methotrexate, TBI – total body irradiation

[11] allo SCT with haploidentical stem cell donor; see chapter 6.1.3.2.1 (Link to German guideline)

15 Authors' Affiliations

Prof. Dr. med. Hubert Schrezenmeier
Universitätsklinikum Ulm
Institut für klinische Transfusionsmedizin
Helmholtzstr. 10
89081 Ulm
h.schrezenmeier@blutspende.de

Univ.-Prof. Dr. med. Tim Henrik Brümmendorf
Universitätsklinikum RWTH Aachen
Medizinische Klinik IV
Klinik für Onkologie, Hämatologie,
Hämostaseologie und Stammzelltransplantation
Pauwelsstr. 30
52074 Aachen
tbruemmendorf@ukaachen.de

Dr. med. Hans Joachim Deeg

Dr. med. Britta Höchsmann
Universitätsklinik Ulm
Institut für Klinische Transfusionsmedizin und Immungenetik
Helmholtzstr. 10
89081 Ulm
b.hoechsmann@blutspende.de

Dr. Sigrid Machherndl-Spandl
Ordensklinikum Linz Elisabethinen
Interne 1 - Hämatologie mit
Stammzelltransplantation, Hämostaseologie
und medizinische Onkologie
Fadingerstr. 1
A-4020 Linz
sigrid.machherndl-spandl@ordensklinikum.at

PD Dr. med. Jens Panse
Universitätsklinikum RWTH Aachen
Medizinische Klinik IV
Klinik für Onkologie, Hämatologie,
Hämostaseologie und Stammzelltransplantation
Pauwelsstr. 30
52074 Aachen
jpanse@ukaachen.de

Prof. Dr. med. Jakob Passweg
Universitätsspital Basel
Hämatologie
Petersgraben 4
CH-4031 Basel
jakob.passweg@usb.ch

Prof. Dr. med. Alexander Röth
Universitätsklinikum Essen
Klinik für Hämatologie
Westdeutsches Tumorzentrum
Hufelandstr. 55
45122 Essen
alexander.roeth@uk-essen.de

Prof. Dr. med. Jörg Schubert
Elblandklinikum Riesa
Innere Medizin II
Hämatologie/Onkologie & Gastroenterologie
Weinbergstr. 8
01589 Riesa
joerg.schubert@elblandkliniken.de

Prof. Dr. med. Bernhard Wörmann
Amb. Gesundheitszentrum der Charité
Campus Virchow-Klinikum
Med. Klinik m.S. Hämatologie & Onkologie
Augustenburger Platz 1
13344 Berlin
bernhard.woermann@charite.de

16 Disclosures

Conflicts of interest can be found in the full German version of the guideline.