

Clinical case, discussion, round table: aplastic anemia with severe infectious complications, allogeneic HSCT: how and when?

Pediatric Perspective

October 2, 2026

M. FAHD



CeRAMIC
centre de référence aplasies médullaires

centre de référence

maladies rares



DISEASE HISTORY

10-year-old girl

May 2025	June 2025	October 2025	November 2025	Dec 2025	Jan 22, 2026
Diagnosis • Autoimmune hepatitis (ASMA 1/160) • Confirmed by liver biopsy • Moderate cytolysis & cholestasis • CBC: Normal Treatment • Corticosteroids 50 mg/d (gradual tapering)	Immunosuppression • Azathioprine introduced at 50 mg/day Genetics • TPMT, NUDT15, ITPA: <i>wild-type</i>	Relapse • Relapse occurs on Corticosteroids 5 mg/day • CBC: Normal Therapy Adjustment • Corticosteroids increased to 25 mg/day • Azathioprine increased to 75 mg/day	Severe Complication • Onset of Pancytopenia (WBC 1430, ANC 330, Plt 86G/L, Hb 9.9) • Liver flare (ALT 883) Bone Marrow Aspirate • Hypocellular marrow • Dyserythropoiesis • Suggestive of marrow toxicity	Treatment Cessation • Azathioprine (Imurel®) STOPPED due to toxicity • Corticosteroids continued at 25 mg/day	Patient Transfer • Transferred to Robert-Debré Hospital for continued care



HEPATITIS



SAA



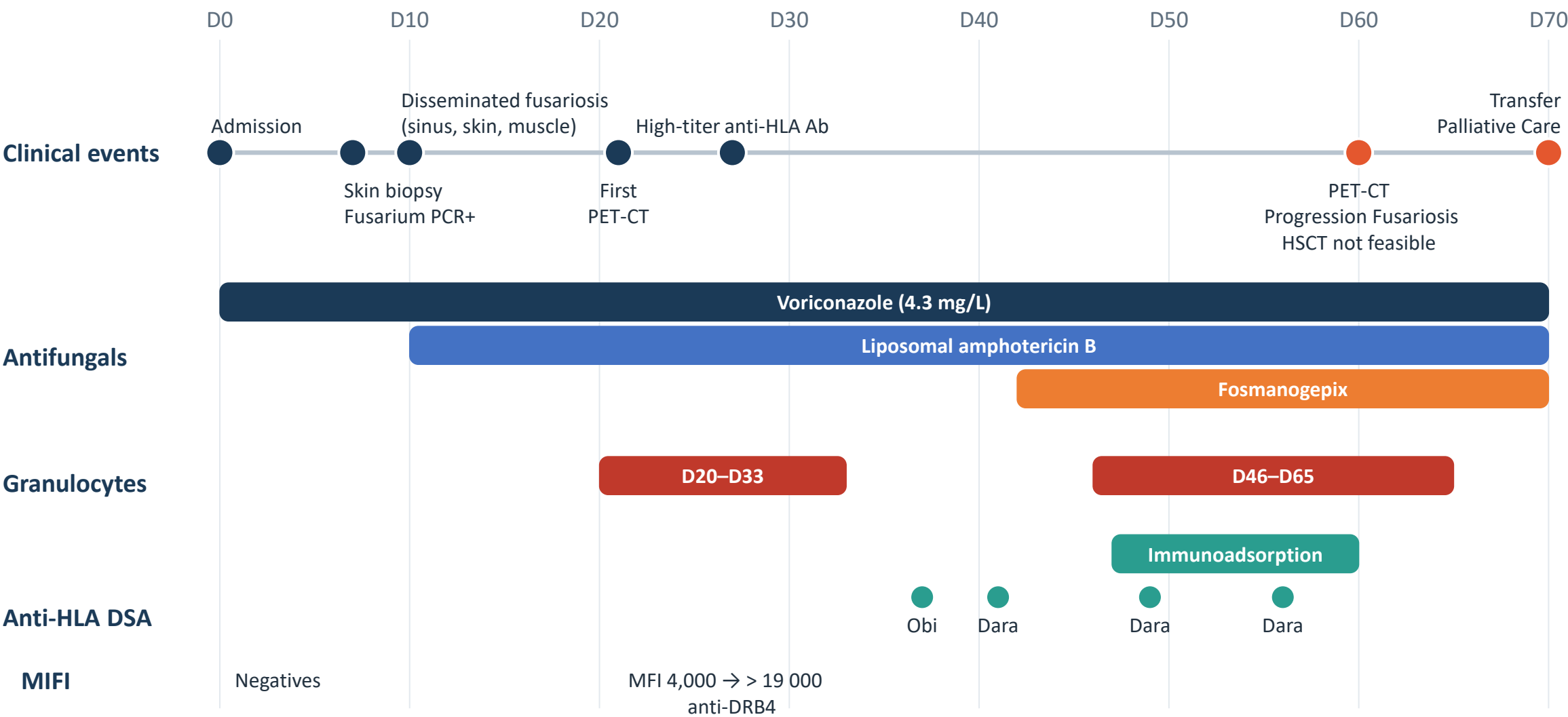
RDB Hospital

APLASTIC ANEMIA WORK-UP & DONORS

Test	Results & Interpretation
Bone Marrow Assessment	• BM: very hypocellular marrow, no blasts. A few stromal elements present, confirming the sample is representative. • Bone marrow biopsy: nil cellularity. Aplastic anemia
Cytogenetics & Molecular Biology	• Karyotype: failed. No monosomy 7 on FISH • NGS for aplastic anemia: negative
Chromosome Fragility & Telomeres	• Telomere length: normal • Chromosome breakage test: failed • FANCD2 test (bone marrow and blood): normal
PNH Clone Screening	• No PNH clone at a sensitivity threshold of 4×10^{-3} on neutrophils and 3% on monocytes.
Other Investigations	• Viral negative • Hemoglobin electrophoresis: normal • Imaging assessment: no anomalies.
Donor search	
Donors	• No siblings • No 10/10 – 9/10 donors • Cord blood (UCB) 6/8 • Healthy haploidentical parents
Anti-HLA Antibodies	• Négatives

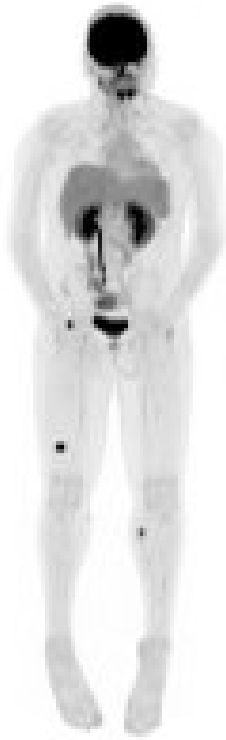
SAA + Hepatitis and disseminated fusariosis

HLA Alloimmunization

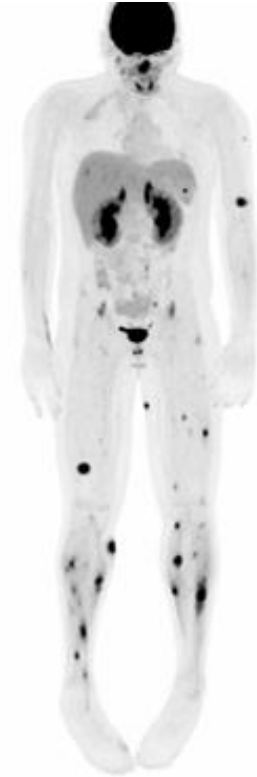


Evolution of fusariosis

M1

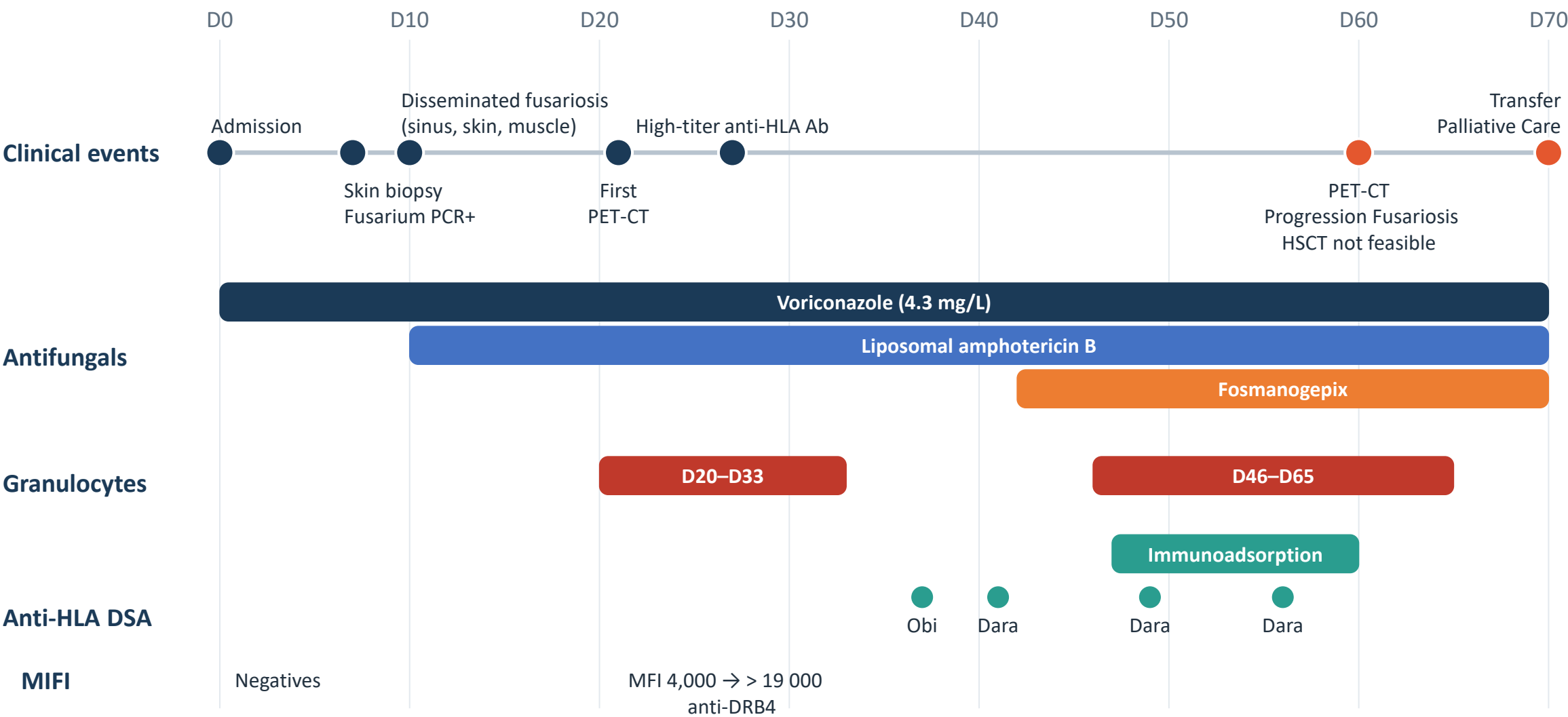


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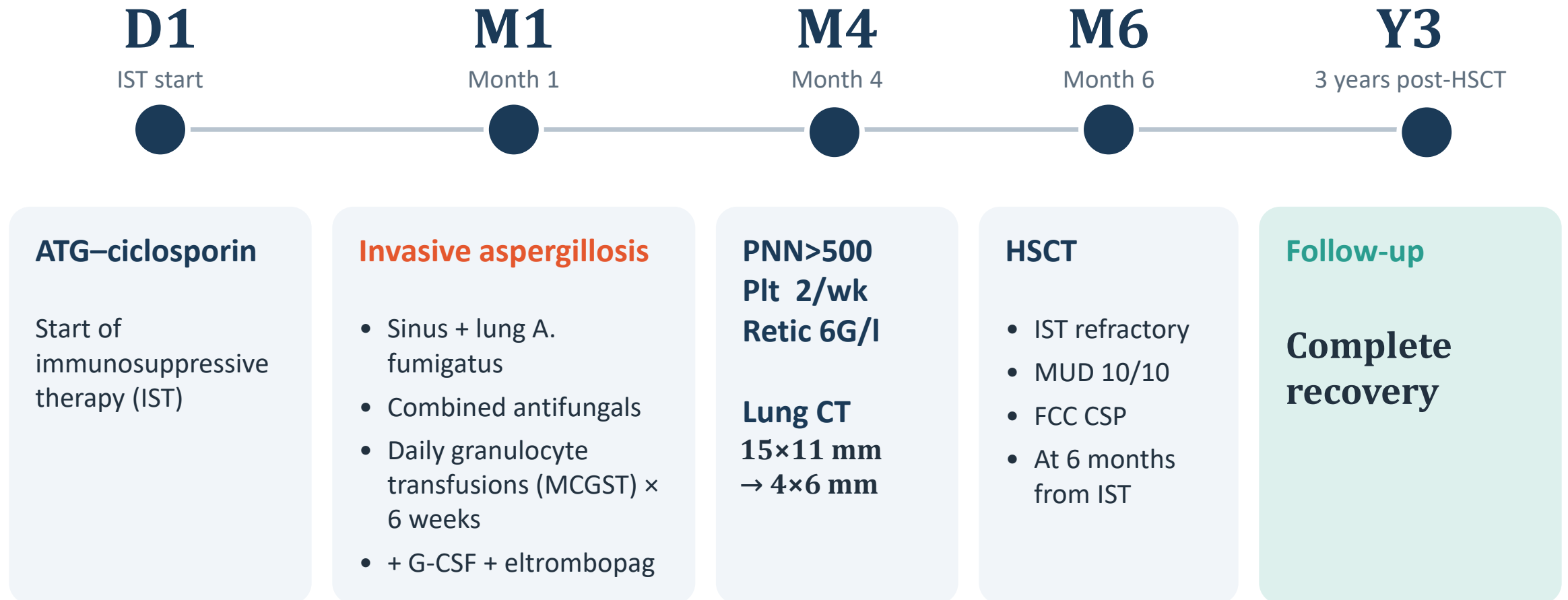
SAA + Hepatitis and disseminated fusariosis

HLA Alloimmunization



12-year-old girl

SAA + Hepatitis and Invasive Aspergillosis



Prevention



- Rapid diagnosis
- Azole prophylaxis
- Hospitalization in a positive-pressure room
- Urgent management of fever
- And urgent therapeutic decision between HSCT and IST+++



PNDS

Severe Infection And Aplastic Anemia

SAA/BMF
RDB 2012-2025

173 patients
93 HSCT

2 fusariosis (deceased)

14 invasive aspergillosis
Including 4 deaths

Case report: HSCT despite active fungal infection

Study	Population	Strategy	Key result
Ikenobe et al.	SAA, disseminated fungal infection after IST	Haploidentical PBSC HSCT	Successful
Zhumatayev et al.	SAA, active mucormycosis	HSCT	Successful
Ma et a.	Child, VSAA, rhino-orbito-cerebral mucormycosis	Allo-HSCT	Successful
Hannemann et al.	Child, AA, invasive fusariosis	Allo-HSCT	Splenic rupture, fungal endocarditis DC

The prognostic impact of previously infectious complications on allogeneic HSCT for patients with severe aplastic anemia; a single-center, retrospective study



Chinese study

Adults and children

SAA with allo-HSCT
with infection (n=66)
and
without infection (n=189)

Characteristics: more patients in the infection group

- were diagnosed with VSAA (59.09% vs 30.69%, $P < 0.001$),
- PBSC grafts more often (89.39% vs 76.72%, $P = 0.042$)

- Multidrug-resistant organism colonization or infection in the infection group (16.7% vs 0.5%, $P < 0.001$)

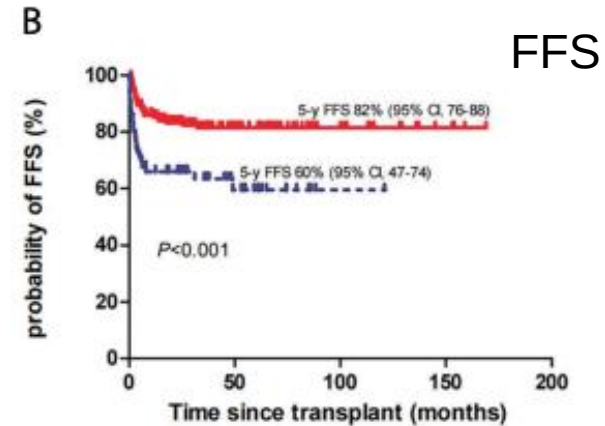
The prognostic impact of previously infectious complications on allogeneic HSCT for patients with severe aplastic anemia; a single-center, retrospective study



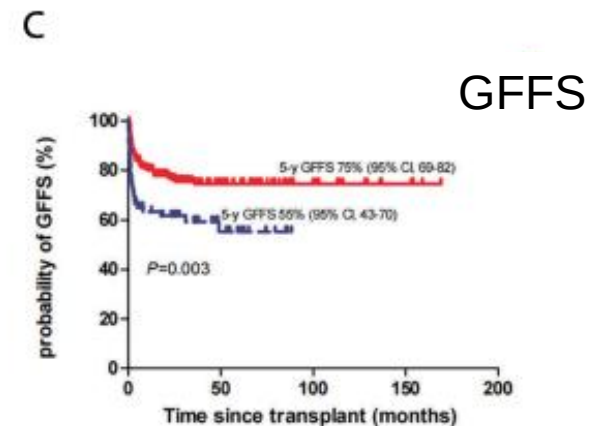
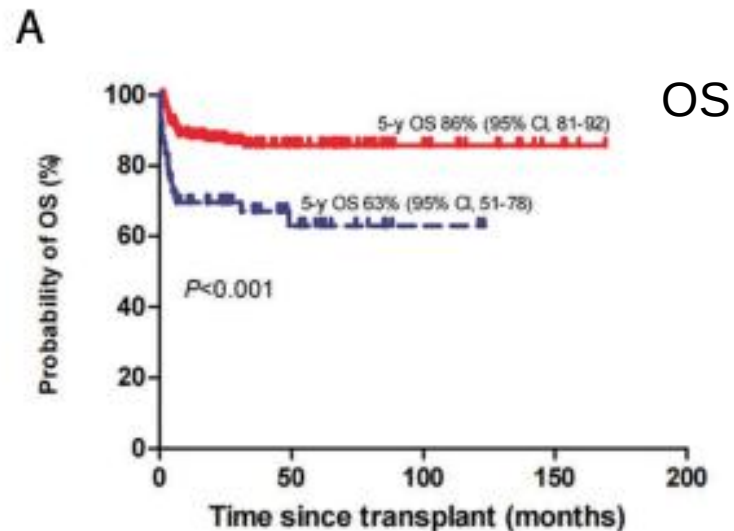
Multivariate analysis

- Haploidentical HSCT
- pre-HSCT anti-infection response
 - partial remission
 - stable disease (SD),

Adverse factors for OS and FFS



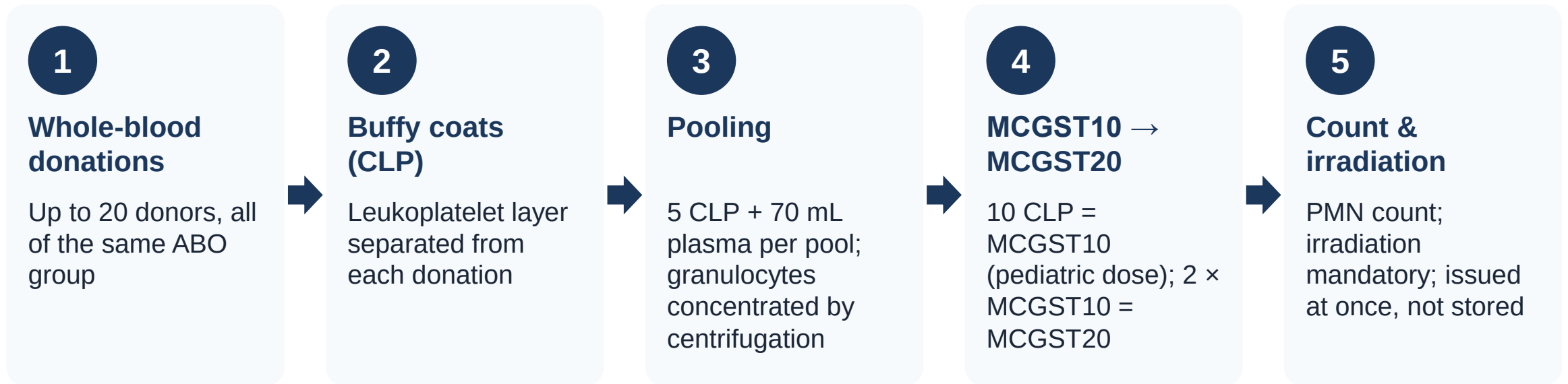
Non Infection Group
Infection Group



Production of MCGST

Pooled granulocyte concentrates from whole blood

Pooled granulocyte concentrate from whole-blood buffy coats: alternative to apheresis (CGA), with no donor stimulation



$\geq 2 \times 10^{10}$

PMN per therapeutic dose

~430 mL

mean volume (MCGST20)

95% → 85%

viability at day 1 → day 2

48 h

MCGST: current status

Available since 2020

Replace the apheresis
granulocyte concentrate (CGA)

Production halted because of a
regulatory impasse requiring off-
label use of corticosteroids.

Since 2026, use of red-cell-
depleted MCGST

In 2025

RDB: 27 MCGST for 4 patients

- 2 SAA
- 2 AL

Île-de-France: 39 MCGST

National: 136 MCGST
(RDB 20%)

Granulocyte transfusions and cohorts

TABLE 1 | Case series of pediatric patients who received therapeutic treatment with GTX* for fungal infections.

References	Underlying conditions	n FI/n total patients	Adverse effects	Clinical response for FI or survival	
Sachs et al. [11]	AA, HO	7/27	Mild fever or chills (7%)	100% response	50% à 100%
Grigull et al. [16]	HSCT, HM	6/32	Fever (> 9%), respiratory distress (5.3%)	67% survival	
Kikuta et al. [17]	HO	2/13	Hypoxia	50% response	
Drewniak et al. [12]	HO, HSCT	13/16	NS	73% response for IA	50% AL, 6 SAA 2003–2013
Graham et al. [13]	HO	8/13	Fever (15%)	50% survival	
Seidel et al. [18]	HO, HSCT	31/69	Fever (14%), chills (3%), airway obstruction (3%)	0.65 ± 0.09 probability of 100 days survival	
Atay et al. [19]	HO, CGD	18/35	No adverse effects	55% response	Single-donor GT (G-CSF + DXM) Median 6
Ozturkmen et al. [14]	HM	4/10	Oliguria, respiratory distress (23%)	50% response	
Diaz et al. [10]	FN	5/18	Respiratory symptoms	80% response	
Nikolajeva et al. [20]	HM, AA, CGD	14/28	Fever (18%), mild respiratory symptoms (11%)	50% radiological improvement, 79% 100 days survival	18/28 alive (64%) 2/10 deaths from infection progression
Kagizmali et al. [22]	FN in HM	26/74	No adverse effects	76% 90 days survival	
Arslantas et al. [21]	HO	NS/60	No adverse effects	85% response, 76% 90 days survival	

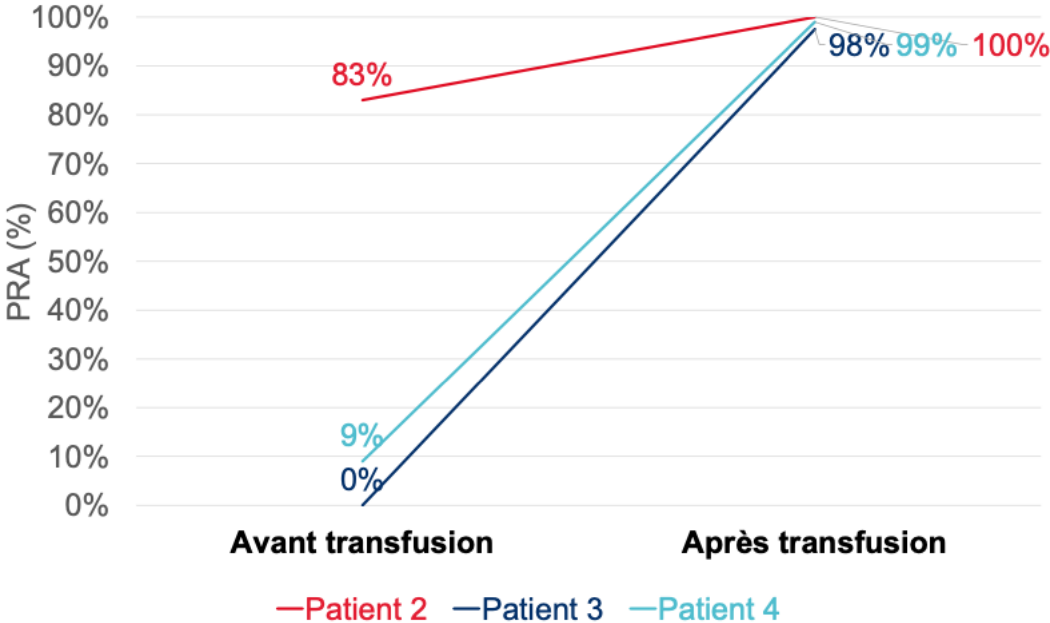
MCGST: risk of HLA alloimmunization?



Allo-immunisation anti-HLA après transfusion de granulocytes chez les patients atteints de granulomatose chronique

P. PEDINI ^{1,2,3}, C. USUREAU ⁴, MJ. APITHY ⁵, T. PAJOT ⁶, V. DE LA TAILLE ³, S. LAPERCHE ³, P. TIBERGHEN ⁷

Patient	Sexe	Age	Infection	CGA ou MCGST	Nombre de transfusions	Allo-immunisation anti HLA (classe I ou II)
1	M	20	Fongique	CGA	60	Non
2	M	7	Fongique	CGA	28	Oui (I + II)
3	M	13	Fongique	CGA	41	Oui (I + II)
4	M	9	Bactérienne	MCGST	14	Oui (I)
5	M	15	Fongique	MCGST	3	Non
6	M	22	Fongique	MCGST	19	Non
7	M	26	Fongique	MCGST	8	Non



Poster SFGM 2025

Other risks: acute pulmonary edema, TRALI, anaphylaxis, fever

Severe infection and AA: HSCT How and when?

1

Strategy at diagnosis

Neutropenia (+/-steroids) rising fungal risk: what should drive the early choice between urgent HSCT and IST, and when must it be made?

2

Donor and DSA

BM or CSP?
DSA treatment?

3

Granulocytes

MCGST: should alloimmunization risk limit their use?

4

Infection control before HSCT

Which criteria justify transplanting during active infection (imaging, PCR clearance)?

Thank you for your attention



Acknowledgments: Aplastic anemia & IBMF French group

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- ***Reference center Lab: Lise LARCHER, Marie PASSET, Nadia VASQUEZ, Jean SOULIER***
- ***Reference center staff: Isabelle BRINDEL Julie CAIGNART, Lynda MAAFA***

