IPOPI 8TH AFRICAN PID PATIENTS' MEETING

14-15 JUNE 2023

CASABLANCA, **MOROCCO**

an **IPOPI** event

8 ÈME RENCONTRE AFRICAINE D'IPOPI POUR **LES PATIENTS ATTEINTS** DE DIP

COLLABORATION

ASID

HAJAR

SUPPORTED BY

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Takeda

IPOPI

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Traduction en temps réel
Real-Time Translation

1. Scanner de QR
Scan the QR:

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IPOPI
8TH AFRICAN
PID PATIENTS'
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CASABLANCA, MOROCCO
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BIENVENUE

WELCOME

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SESSION 4

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SESSION 4:

Traitement et prise en charge des DIP en Afrique
Treatment and care of PIDs in Africa

Modérateur | Moderator: Johan Prevot
Intervenants | Speakers: Dr. Anne Barasa; Houda Chadil; Dr. Monia Ouederni; Prof. Aziz Bousfiha

COLLABORATION

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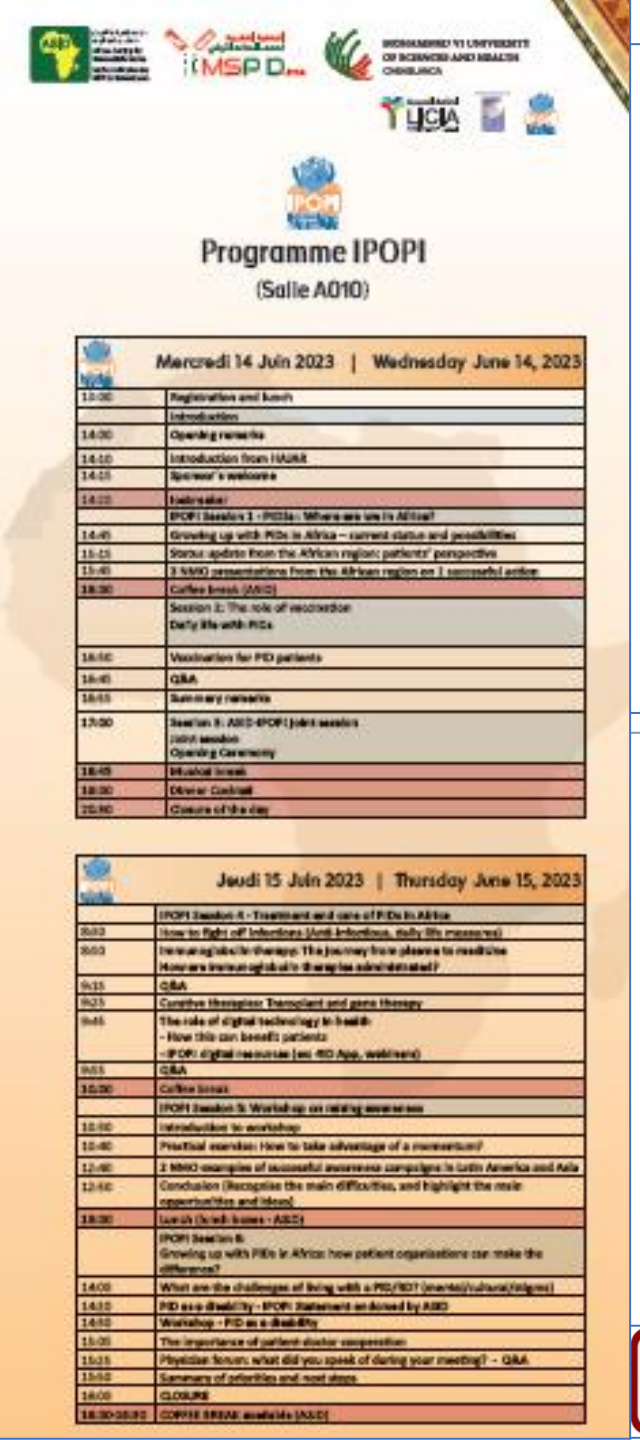
Vaccination des patients atteints de salpingite

Vaccination for PID patients

Prof. Aziz Bousfiha



Programme IPOPI

(Salle A010)

Mercredi 14 Juin 2023 | Wednesday June 14, 2023	
13:00	Registration and lunch
	Introduction
14:00	Opening remarks
14:10	Introduction from IAUAA
14:15	Sponsor's welcome
14:20	Icebreaker
	IPOPI Session 1 - PIDDs: Where are we in Africa?
14:45	Growing up with PIDs in Africa – current status and possibilities
15:15	Status update from the African region: patients' perspective
15:45	3 MMO presentations from the African region on 3 successful action
16:30	Coffee break (ASID)
	Session 2: The role of vaccination Daily life with PIDs
16:50	Vaccination for PID patients
16:40	Q&A
16:55	Summary remarks
17:30	Section 3: ASID-IPOPI joint session Joint session Opening Ceremony
18:45	Musical break
19:30	Dinner Cocktail
20:30	Closure of the day

Jeudi 15 Juin 2023 | Thursday June 15, 2023	
	IPOPI Session 4 - Treatment and care of PIDs in Africa
8:02	How to fight off infections (Anti-infectious, daily life measures)
8:02	Immunoglobulin therapy: The journey from plasma to medicine How are immunoglobulin therapies administered?
9:15	Q&A
9:25	Curative therapies: Transplant and gene therapy
9:45	The role of digital technology in health - How this can benefit patients - IPOPI digital resources (ex: IEI App, webinars)
9:55	Q&A
10:20	Coffee break
	IPOPI Session 5: Workshop on raising awareness
11:00	Introduction to workshop
11:40	Practical exercise: How to take advantage of a momentum?
12:40	2 MMO examples of successful awareness campaigns in Latin America and Asia
12:50	Conclusion (Recognise the main difficulties, and highlight the main opportunities and ideas)
13:30	Lunch (Joint Session - ASID)
	IPOPI Session 6: Growing up with PIDs in Africa: how patient organisations can make the difference?
14:00	What are the challenges of living with a PID/IEI (genetic/cultural/stigma)
14:10	PID as a disability - IPOPI Statement endorsed by ASID
14:50	Workshop - PID as a disability
15:20	The importance of patient-doctor cooperation
15:35	Physician forum: what did you speak of during your meeting? - Q&A
15:50	Summary of priorities and next steps
16:00	CLOSURE
16:30-20:30	COFFEE BREAK available (ASID)

Vaccination des Familles PIDs

Vaccination of PID Families

تلقيح عائلات ضمأ

أحمد عزيز بوصفيحة

Pr Ahmed Aziz BOUSFIHA – CHU Ibn Rochd LICIA-FMPC - UH2 - Casablanca

قسم الأمراض التعفنية و المناعية لدى الطفل ،

المشفى الجامعي ابن رشد - جامعة الحسن الثاني - الدار البيضاء

كلية الطب و الصيدلة، جامعة الحسن الثاني

مختبر المناعة السريرية و الإلتهاب و الأرجية LICIA

Vital à faire, URGENT ! إجراءات حيوية، مستعجل

1. Vacciner votre entourage لقحوا أسركم
2. Comprendre qu’il n’ya pas que vos anticorps qui vous protègent حمايتكم ليست فقط بالضدأجسام
3. Ayez une culture vaccinale اكتسبوا ثقافة تلقيحية

Vacciner votre entourage لقحوا أسركم

- Car Efficacité des vaccins est réduite chez vous لأن الإستجابة التلقيحية عندكم منخفضة
- Qui ? من ؟
  - Les personnes et les animaux à domicile الأشخاص و الحيوانات في منزلكم
  - Les amis les plus proches أصدقاؤكم الأقربين
  - A l’école au travail à l’hôpital في المدرسة و العمل و المشفى

اللقاح يحمي الفرد ...

عندما يتم تطعيم المجتمع المحلي، فإنّ جميع الأفراد يتمتعون بالحماية، حتى أولئك الذين لا يمكن تطعيمهم بسبب إصابتهم بحالات صحية كامنة.

منظمة الصحة العالمية

https://www.who.int/ar

Comprendre qu’il n’ya pas que vos anticorps qui vous protègent
حمايتكم ليست فقط بضدأجسامكم

- L’Immunité: Anticorps + Lymphocytes + Macrophages المناعة = الضدأجسام + اللمفاويات + البلعوميات
- L’efficacité vaccinale chez vous est faible mais non nulle الفعالية اللقاحية عندكم منخفضة لكن ليست منعدمة
- Il faut profiter au maximum des vaccins يجب الاستفادة من اللقاحات أكثر ما يمكن

Ayez une culture vaccinale اكتسبوا ثقافة تلقيحية

- La meilleure découverte après l'eau potable أحسن اكتشاف بعد الماء الصالح للشرب
- Les types de Vaccins : أنواع اللقاحات
  - Vivant atténués: Potentiellement nocifs pour vous الحي الموهن : يمكنه أن يضركم
  - Aucun risque pour vous: هذه اللقاحات لا تشكل خطرا عليكم
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  - Les rappels (DTCoq Polio) التذكير : الخناق الكزاز العواية الشلل
  - Contre Pneumocoque, Grippe, Covid, HPV, HVA, HVB
  - المكورة الرئوية الزكام كوفيد سرطان عنق الرحم ذات الكبد أ و ب
- Pour votre Entourage : لأقاربكم
  - Comme vous: مثلكم
  - En plus vaccins vivants atténués: ROR, Varicelle و كذالك الحصبة الحميراء النكاف الحماق

♡ اللقاحات

ضرورية لمكافحة الأمراض واستئصالها

"عمر مديد للجميع"

منظمة الصحة العالمية
المكتب الإقليمي لشرق المتوسط

Royaume du Maroc
Ministère de la Santé
et de la Protection Sociale

المملكة المغربية
وزارة الصحة والحماية الاجتماعية

التلقيح، أمل في الحياة

La vaccination, un espoir pour la vie

الأسبوع الوطني للتلقيح

من 25 إلى 30 أبريل 2022

وزارة الصحة والحماية الاجتماعية
Ministère de la Santé et de la Protection sociale

نونبر 2016 - صفر 1438 | العدد 15

المجلة الصحية المغربية

مجلة تصدرها الجمعية المغربية للتواصل الصحي

ملف العدد

التلقيح

المؤتمر المغاربي الأول والوطني التاسع
لطب الصناعة الأولي

1e Maghreb and 9e Moroccan
PID Congress

أبريل 2018 - رجب 1439 | العدد 20

المجلة الصحية المغربية

Moroccan Health Journal

مجلة تصدرها الجمعية المغربية للتواصل الصحي

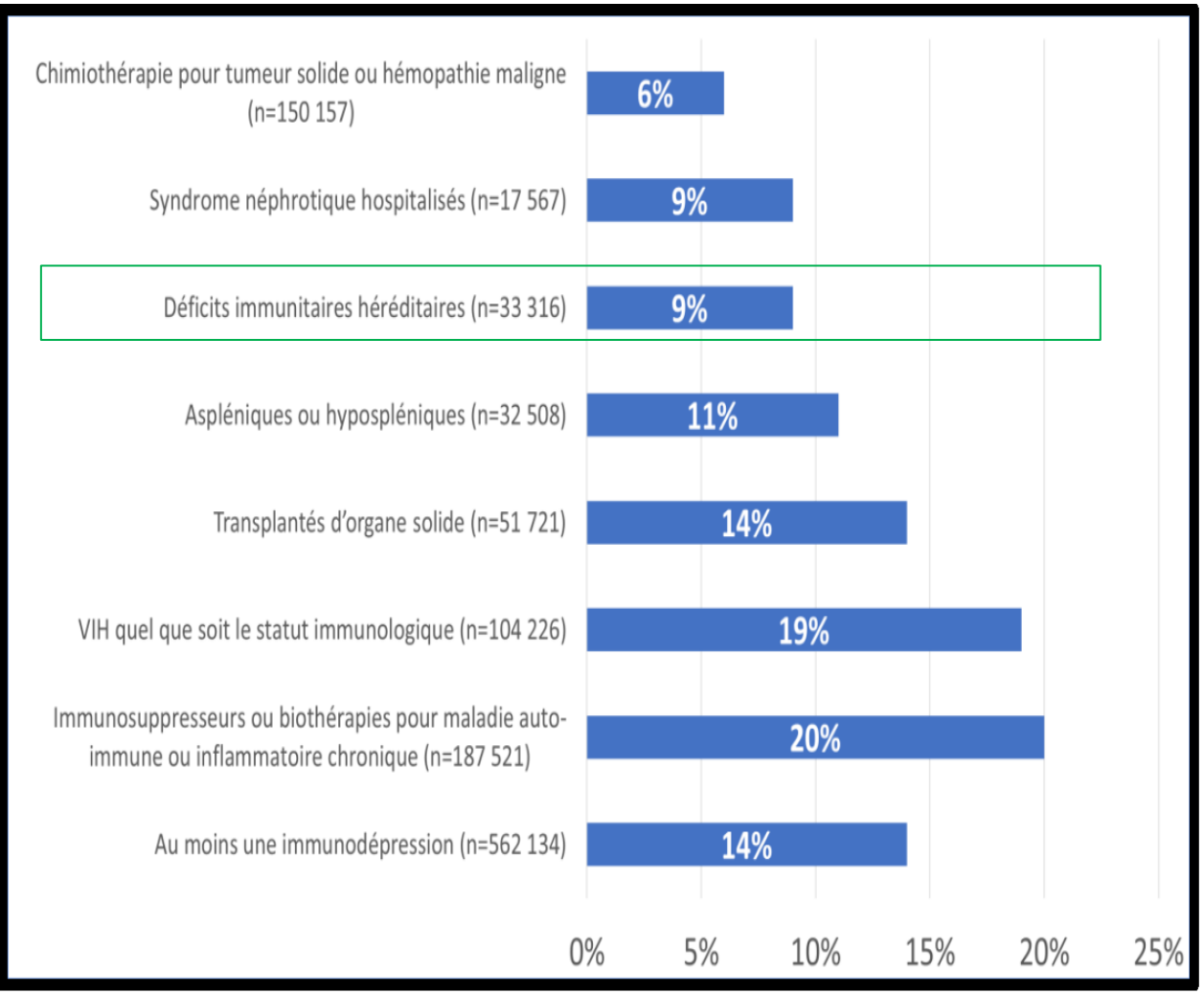
ملف خاص عن

التلقيح

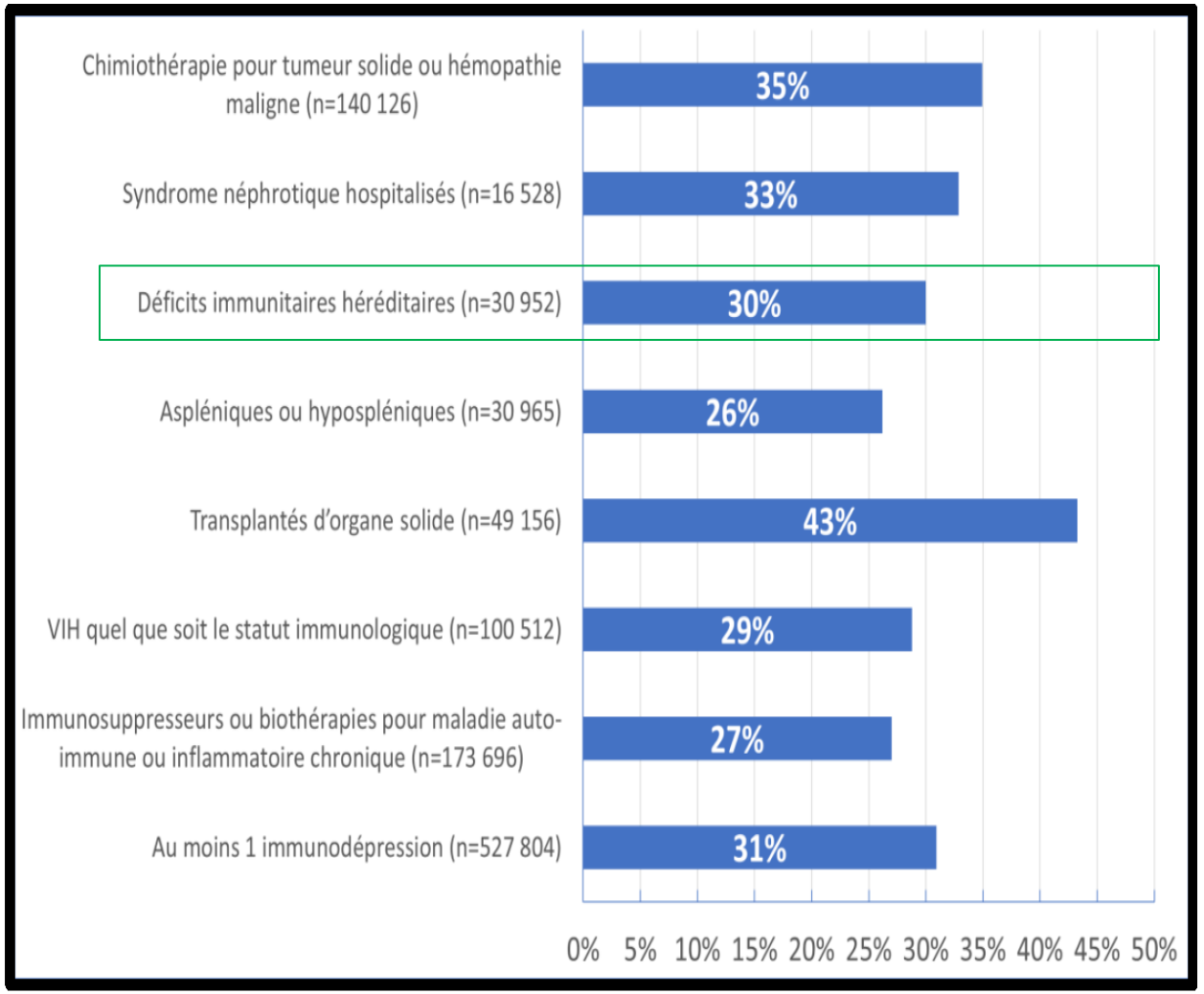
30 سنة

Les Patients Adultes ID sont Peu Vaccinés Contre Grippe et Pneumo !

Contre le Pneumo, 2017



Contre la Grippe, 2017



Benjamin Wyplosz. Couvertures vaccinales des adultes à risque (étude COVARISQ) et impact de la COVID sur les attitudes de prévention. JNI 2020

https://www.infectiologie.com/UserFiles/File/jni/2020/sympo/jni2020-sy11-03-vaccin-wyplosz.pdf

Hôpital d'Enfants de Casablanca, 500 Coqueluches, 2021-2019[1]

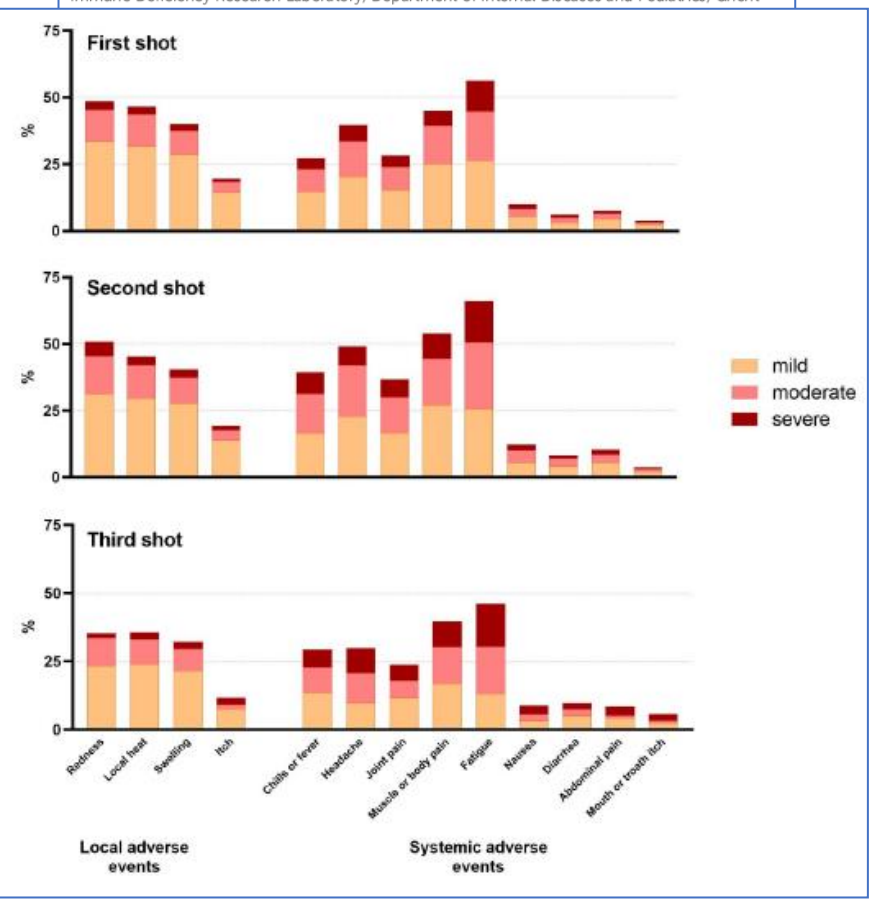
- **Age moyen :**
  - 72 jours [19 jours ,18 mois]
  - < 3 mois : 77 %
  - Non ou incomplètement vaccinés: 92 %
- **Formes compliquées : 8,6%**
  - Apnées : 32 cas 6,4 %
  - Détresse respiratoires : 1,2 %
  - Arrêt cardio-respiratoire : 0,8 %
  - Convulsions: 3 cas, 0,6 %
- **Contaminateur :**
  - Entourage : 48,6%
  - Maman : 33,4%

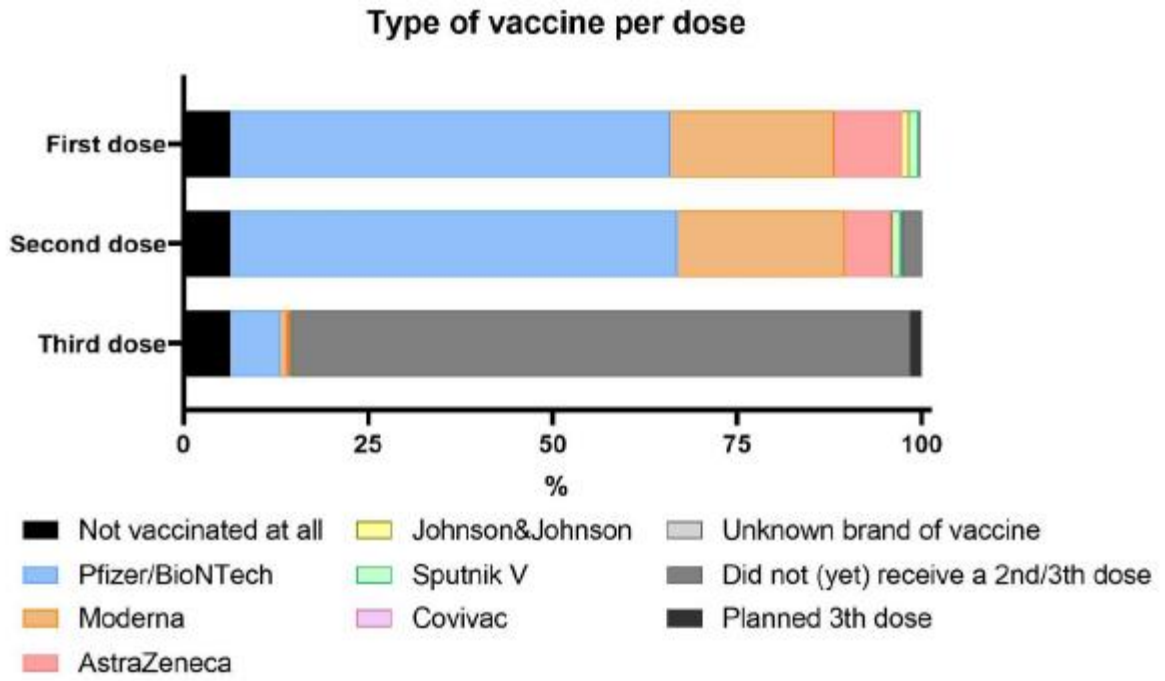
1- H. Saidi, SOMIPEV.MA 2020

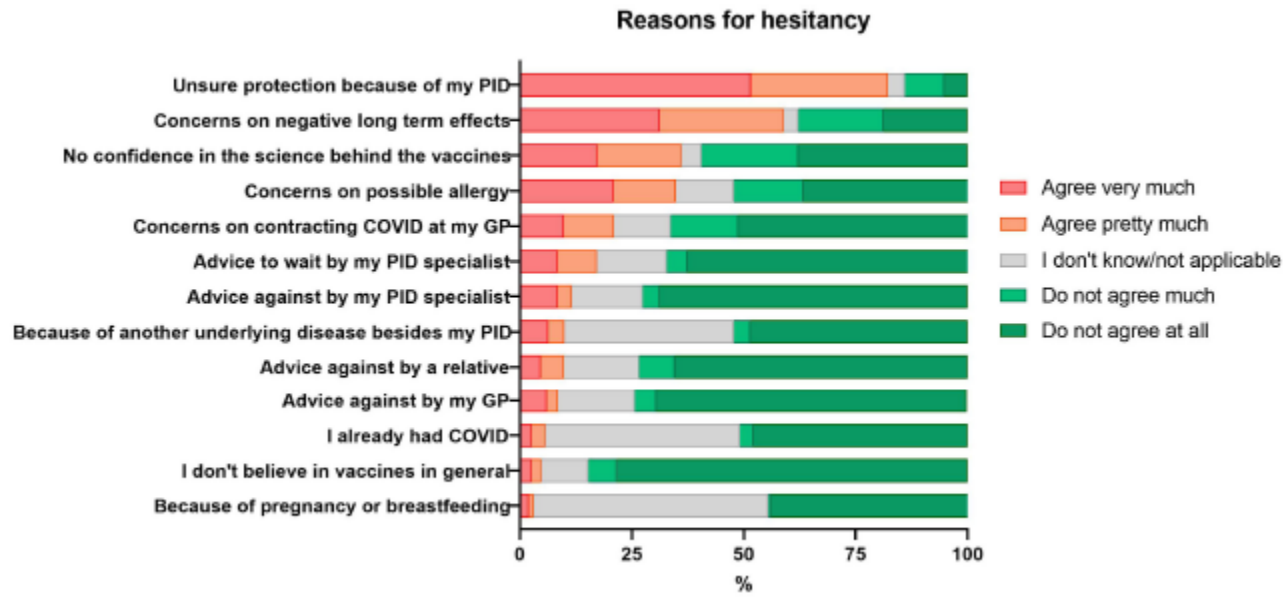
COVID-19 vaccination in patients with primary immunodeficiencies: an international survey on patient vaccine hesitancy and self-reported adverse events

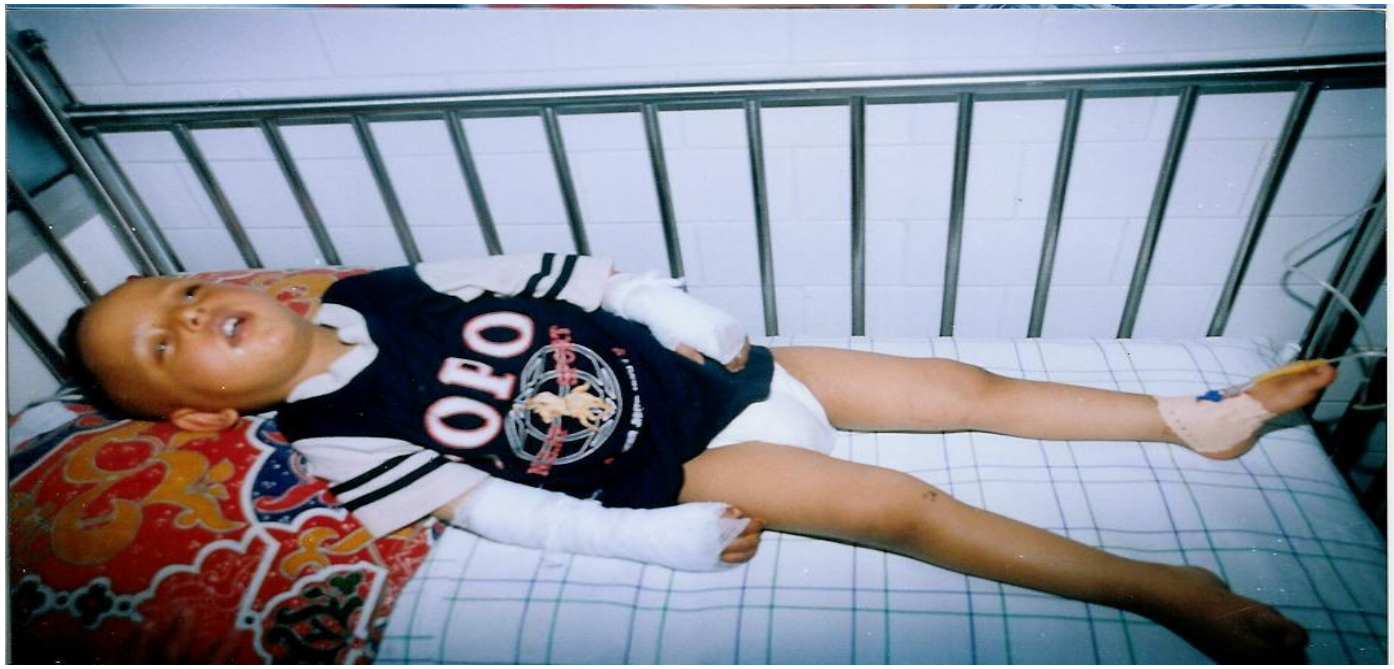
Martine Pergent[1*], Filomeen Haerynck[2,3], Levi Hoste[2,3] and Ann Gardulf[4,5,6]

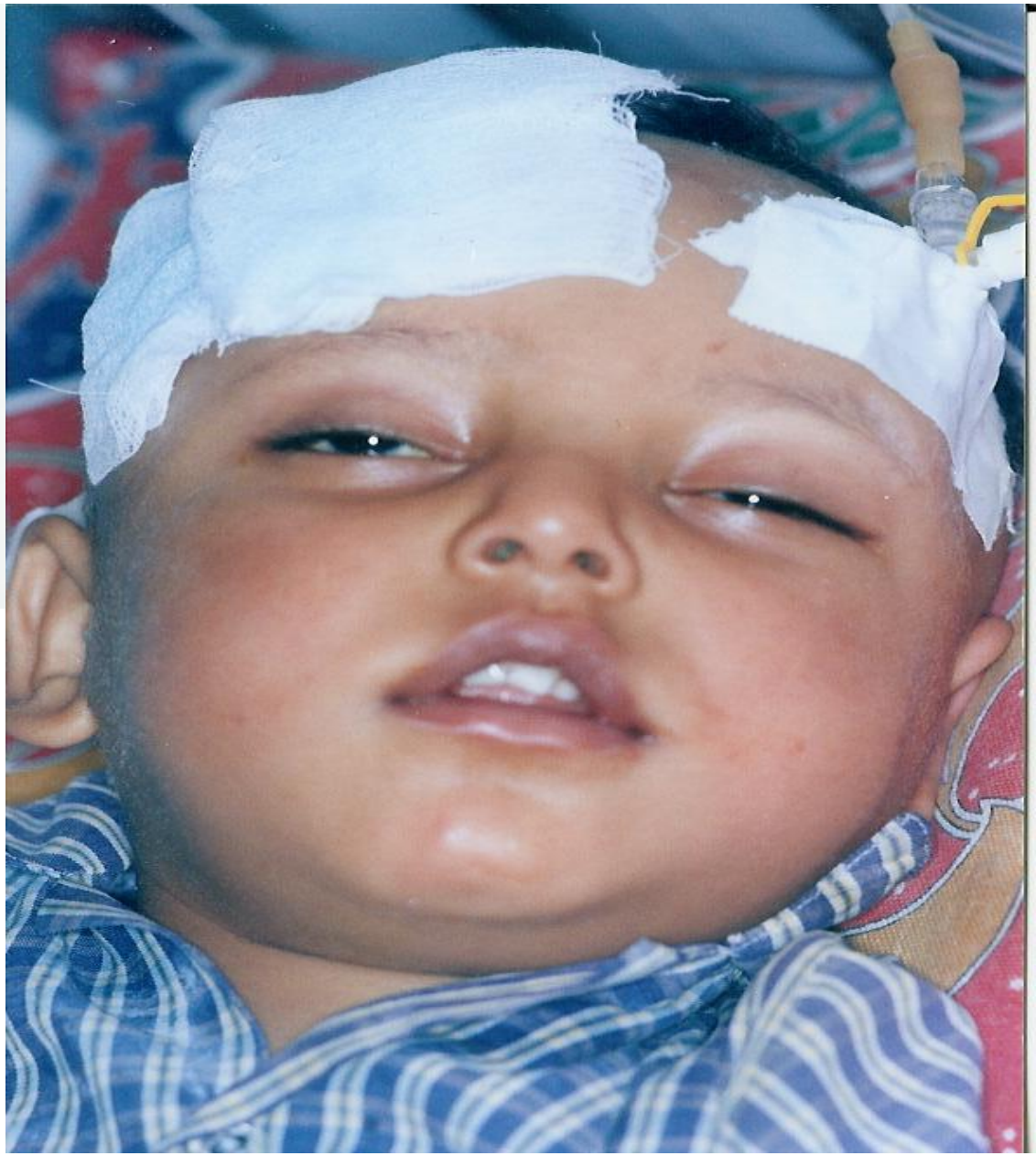
[1]The International Patient Organisation for Primary Immunodeficiencies, Brussels, Belgium, [2]Primary Immune Deficiency Research Laboratory, Department of Internal Diseases and Pediatrics, Ghent











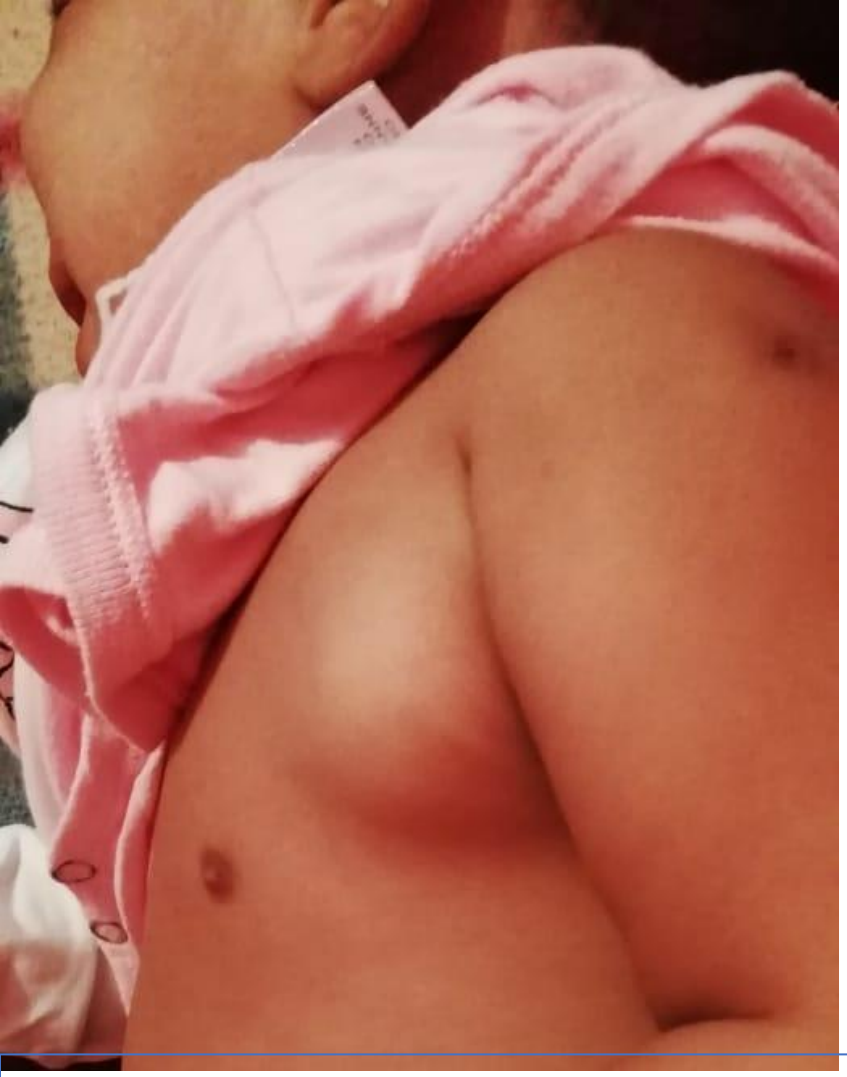
عثمان 18 شهرا

يرقان ثم غيبوبة

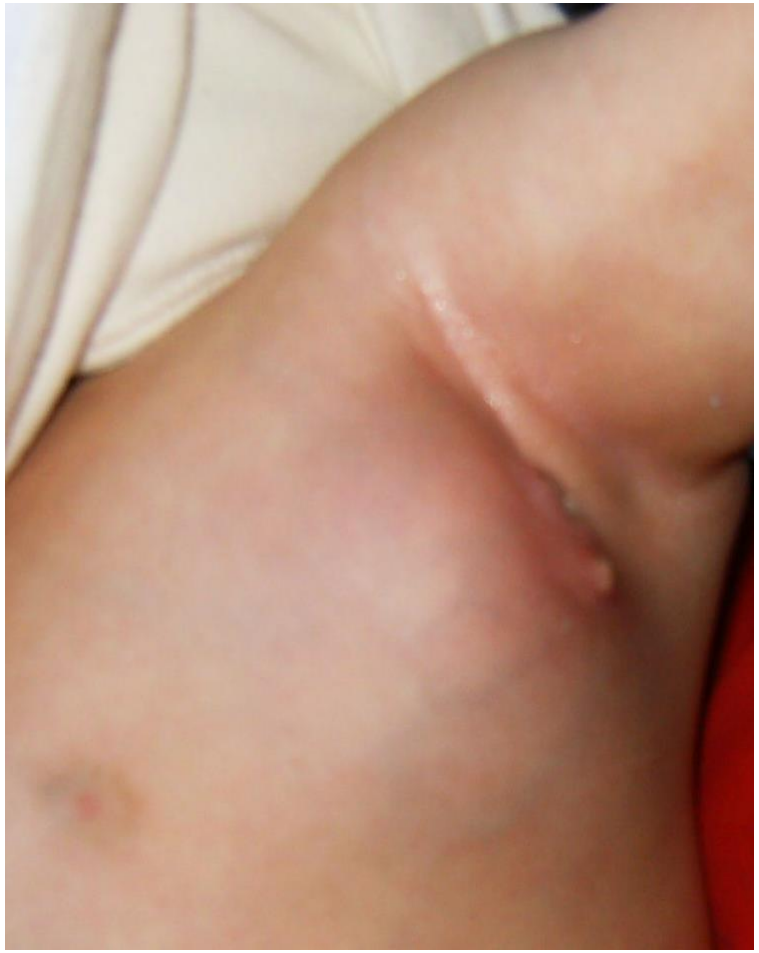
Tansaminases : 5000 , TP : 14 %, IgM HVA +

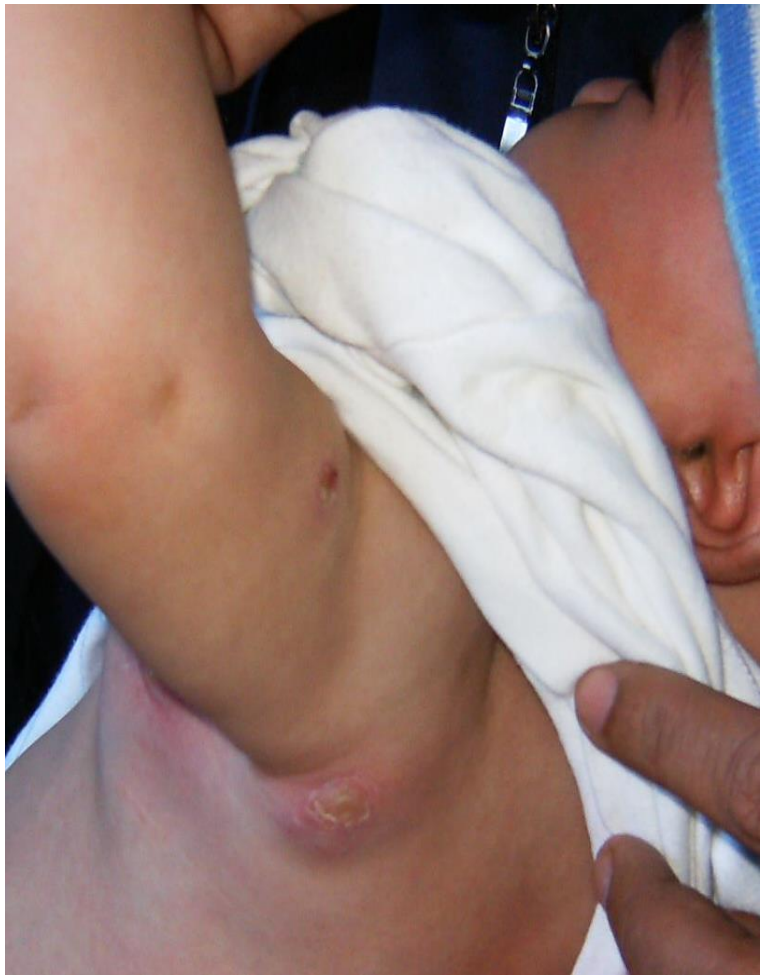
IL18BP Deficiency

توفي في اليوم الثالث عشر



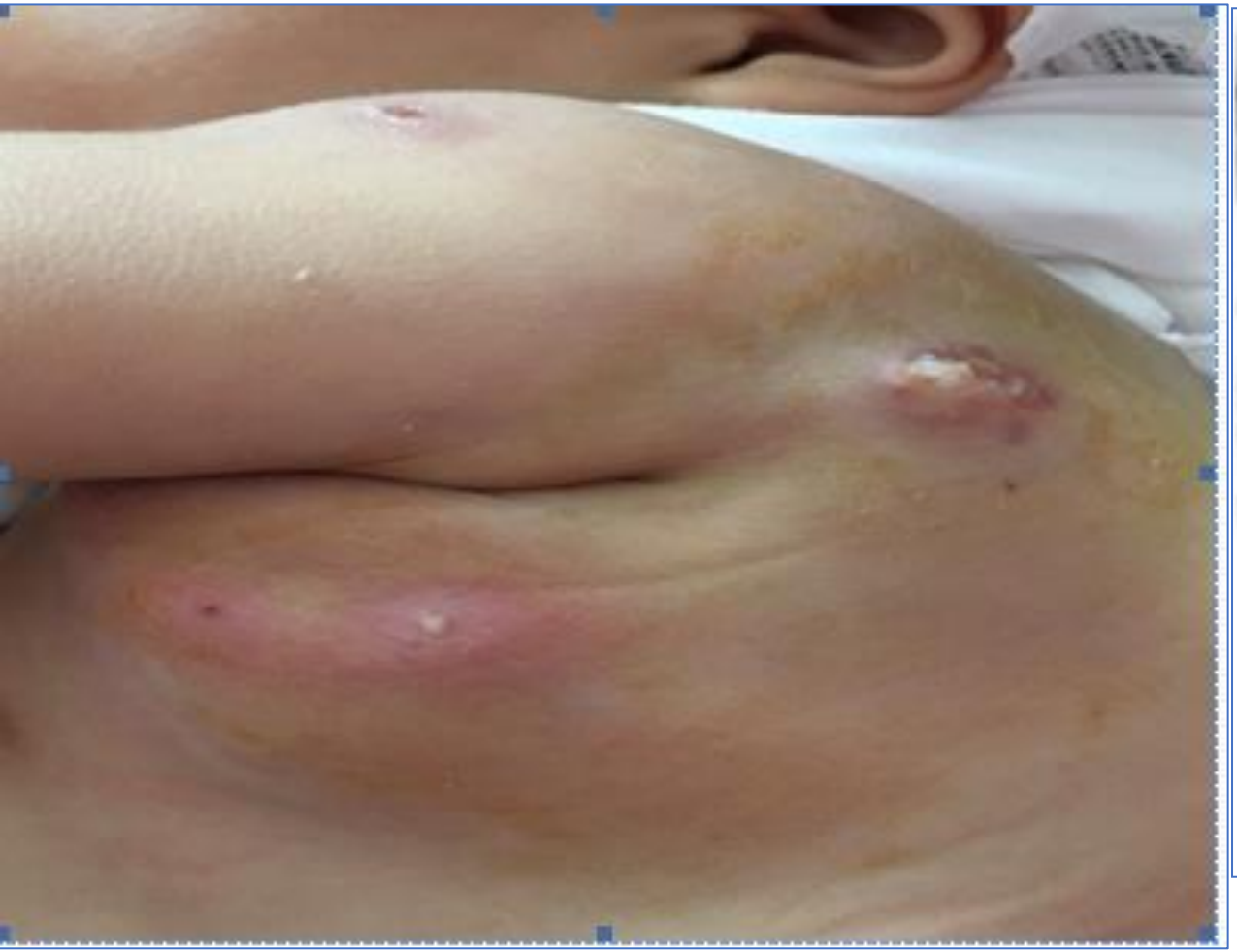
Fille, 11 mois
BAAR + au niveau
Adénomectomie Axillaire G.
SCID T-B+NK- Jack3D.

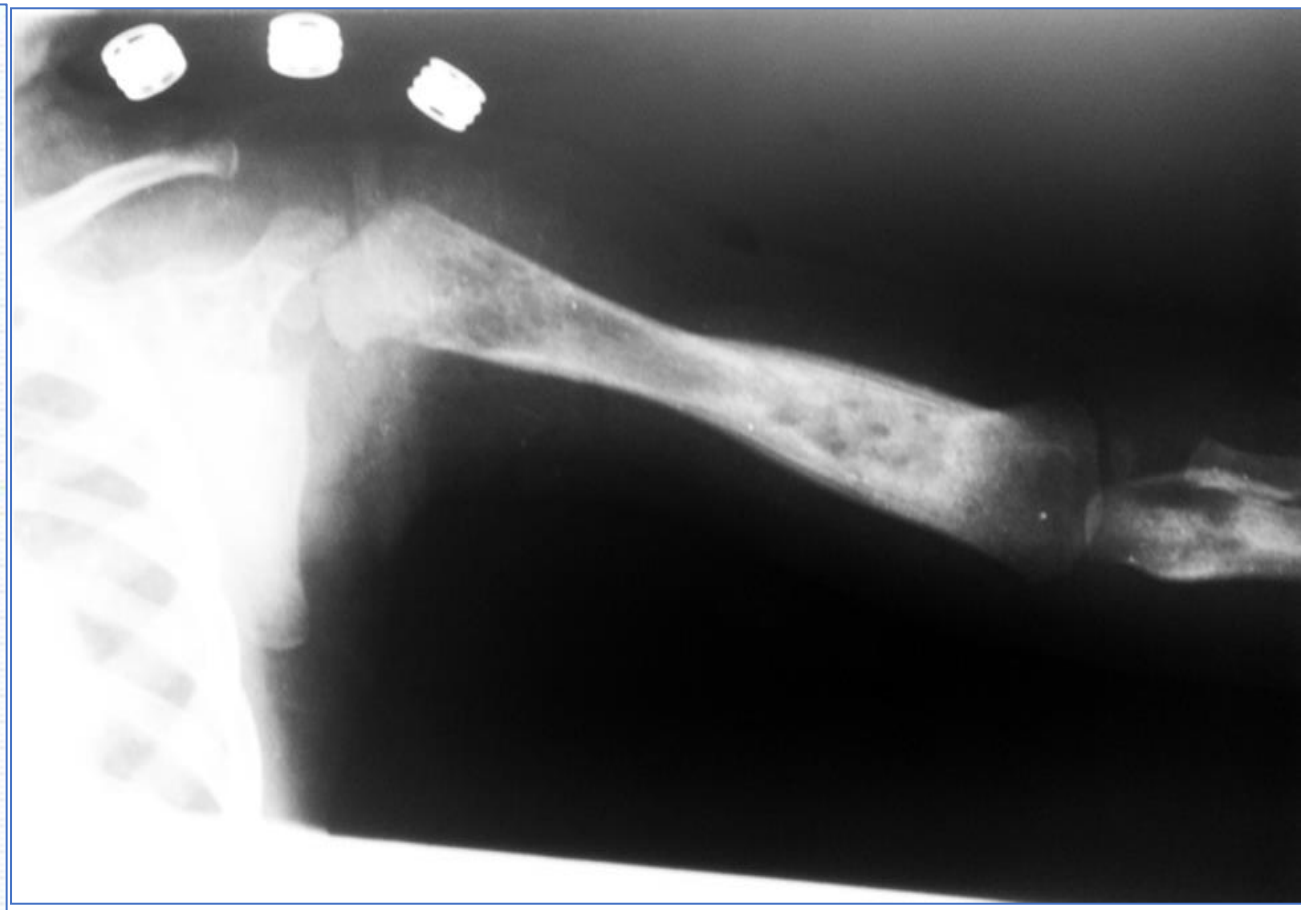




- Garçon, 3 mois, Consanguinité +
- BCGosis,
- MSMD: Mutation sur ILRB1

Fille 6 mois, BCGosis
D. IL12RB1





انخفاض معدلات الإصابة بالدرن الرئوي يؤخر مواعيد اللقاح

الأربعاء - 10 يوليو 2019

أحمد الزايدي - الطائف

A A

Vital à faire, URGENT ! إجراءات حيوية، مستعجل

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Conclusions

- Militez pour vous vacciner et votre entourage لنناضل حتى نستفيد من تلقيحنا و أسرنا
- Demander toujours avis à vos médecins دائما استشيروا
- Nous vous aimons très fort ! نحبكم

Comment lutter contre les infections (mesures anti-infectieuses, vie quotidienne)

How to fight off infections (Anti-infectious, daily life measures)

Dr. Anne Barasa | Kenya

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Comment lutter contre les infections

Dr. Anne K. Barasa
University of Nairobi
Kenya

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Introduction

Les patients atteints de DIP sont prédisposés aux infections car ils sont nés avec un élément de leur système immunitaire qui dysfonctionne.

Infections plus fréquentes que d'habitude

Infections graves ou difficiles à traiter

Infections causées par des organismes inhabituels

Les piliers de la prévention des infections

Mesures anti-infectieuses

Mesures prophylactiques mises en place par le personnel de santé

Prophylaxie antimicrobienne
Substitution en immunoglobulines
Vaccination

Mesures de la vie quotidienne

Responsabilité du soignant et/ou du patient

Pratiques d'hygiène et soins personnels

Prophylaxie antimicrobienne

Antibiotiques, antiviraux et antifongiques prescrits à long terme pour protéger contre les infections bactériennes, virales ou fongiques.

Le choix de l'antimicrobien est guidé par :

- le risque d'infection du patient dans le cadre de son DIP
- le risque individuel d'infection, en fonction des antécédents du patient
- la prise en compte des effets secondaires
- le risque de résistance antimicrobienne

Prophylaxie antimicrobienne

Dans les déficits immunitaires liés aux cellules T :

- *SCID* - Infections virales, bactériennes et fongiques
- La prophylaxie doit commencer tôt et se poursuivre jusqu'au traitement définitif, s'il est effectué (greffe de cellules souches).
- Antiviraux, antifongiques, antibiotiques
- Mesures d'isolement protecteur

Prophylaxie antimicrobienne

Dans les immunodéficiences des cellules T :

- Substitution en immunoglobulines
- Les produits sanguins doivent être CMV négatifs et irradiés.
- Si le vaccin BCG a été administré, une prophylaxie doit être mise en place pour prévenir une infection BCG disséminée - maintenue jusqu'à la reconstitution immunologique.

Prophylaxie antimicrobienne

Dans les Troubles phagocytaires :

Granulomatose septique chronique

- Infections bactériennes et fongiques récurrentes et graves
- Prophylaxie antibiotique et antifongique

Susceptibilité mendélienne aux infections mycobactériennes

- Prophylaxie contre les mycobactéries environnementales

Prophylaxie antimicrobienne

Dans les déficits en anticorps primitifs :

- Susceptible de contracter des infections bactériennes des sinus ou des poumons chroniques ou récurrentes et des entérovirus.
- Les personnes atteintes d'une maladie grave doivent recevoir des immunoglobulines - traitement le plus important.
- Antibiotiques prophylactiques si >3 infections récurrentes par an ; ou toute infection sévère, malgré une substitution adéquate en immunoglobulines.

Autre prophylaxie anti-infectieuse

Substitution en immunoglobulines

- Prescrites en fonction de la situation individuelle du patient.

Vaccination

- Opportune et appropriée en fonction de l'état et de l'âge du patient
- Les vaccins qui seront bénéfiques ne doivent pas être évités
- Prudence avec les vaccins vivants atténués
- La vaccination des membres de la famille et des contacts étroits est fortement recommandée, afin de réduire le risque d'infection pour le patient.

Traiter les autres causes

- Certaines conditions médicales non infectieuses peuvent contribuer à une infection récurrente
- Il convient de les identifier et de les traiter
- Par exemple, les affections allergiques :
  - Les rhinites allergiques et l'asthme peuvent entraîner des infections des voies respiratoires.
  - L'eczéma peut entraîner des infections cutanées

Hygiène des mains

Bonnes pratiques d'hygiène

Hygiène dentaire

- Brossage des dents au moins deux fois par jour
- Visites régulières chez le dentiste

Étiquette de la toux et de l'éternuement

Pour les patients et les membres de leur famille

- Recueillir la toux et les éternuements dans un mouchoir en papier pour éviter la propagation des infections.
- Utiliser des mouchoirs jetables

Hygiène et soins personnels

- Les coupures et les plaies doivent être nettoyées et pansées rapidement.
- L'espace de vie du domicile/patient doit rester propre.
- Les jouets des enfants doivent être nettoyés régulièrement.
- Les membres de la famille, les amis et les soignants doivent être informés des mesures de contrôle des infections.

La nutrition

- Alimentation saine et équilibrée
- Éviter les aliments crus ou insuffisamment cuits (viandes, œufs, fromages)
- Suppléments généralement inutiles ; consultez votre médecin avant d'en prendre.
- Éviter de boire de l'eau d'origine inconnue
- Ne partagez pas d'aliments ou de boissons avec d'autres personnes.
- Manipuler correctement les aliments pour éviter les intoxications alimentaires

Exercice/activité physique

- Rester en forme est important pour la santé en général - exercice régulier.
- Demandez à votre médecin quelles sont les activités qui vous conviennent.
- Évitez les sports de contact si le DIP affecte la capacité de coagulation du sang.
- Évitez de nager dans les lacs et les étangs (en particulier pour les patients souffrant de déficiences phagocytaires).

Repos adéquat et gestion du stress

- Dormir suffisamment.
- S'endormir et se réveiller à la même heure tous les jours.
- Veillez à dormir le même nombre d'heures chaque nuit.
- Participez à des activités relaxantes qui peuvent réduire le stress, par exemple les loisirs, la méditation (trouvez ce qui vous convient).

Éviter les expositions

- Personnes souffrant d'infections contagieuses
  - Toux, rhumes...
  - Retrait des enfants de l'école pendant les périodes d'épidémies de varicelle, de rougeole, de maladies diarrhéiques aiguës...
- Lieux bondés
- Zones fumeurs
  - Les personnes atteintes de DIP ne doivent pas fumer.
  - Les parents d'enfants atteints de DIP ne doivent pas fumer.

Éviter les expositions

Les patients souffrant de déficits immunitaires primitifs tels que les troubles phagocytaires doivent éviter les moisissures.

- Minimiser le contact avec la saleté, la poussière ou la terre
- Éviter les travaux de jardinage qui impliquent de creuser ou d'entrer en contact avec des plantes ou des arbres en décomposition
- Éviter les zones de construction/rénovation
- Contact avec les animaux de compagnie et autres animaux

Autres mesures générales

- Pratiquer des rapports sexuels protégés pour éviter les infections sexuellement transmissibles, par exemple en utilisant des préservatifs.
- Demander l'avis d'un médecin avant d'effectuer un piercing, un tatouage ou une procédure similaire.
- Tous les professionnels de la santé consultés doivent être informés du diagnostic ou de l'affection sous-jacente.
  - Par exemple, éviter les injections intramusculaires.
  - Si une intervention chirurgicale est nécessaire, il est important que le chirurgien soit informé afin que des mesures soient prises pour prévenir les complications infectieuses.

Précautions à prendre en cas de voyage

- Préoccupations:
  - Infections émergentes
  - Infections propres à une région géographique spécifique
  - Risques liés au processus de voyage
- Discutez de vos projets de voyage avec votre médecin et/ou un spécialiste des maladies infectieuses.
  - Il vous conseillera sur les questions de prévention et sur la nécessité de se faire vacciner.
  - Il peut organiser toute thérapie programmée nécessaire pendant le voyage.
  - Si un certificat de fièvre jaune est exigé, un certificat d'exemption peut être nécessaire.

Précautions à prendre en cas de voyage

- Il peut être nécessaire d'éviter les pays où le risque d'infection est élevé.
- Veillez à disposer d'une assurance médicale et d'une assurance voyage adéquates.
- Porter une déclaration expliquant leur état de santé et le but des médicaments ou de l'équipement qu'ils portent sur eux.
- Faire preuve d'une grande vigilance en matière d'hygiène alimentaire.
- Éviter de boire l'eau du robinet (y compris les glaçons).
- Précautions à prendre pour éviter les piqûres d'insectes

Collaboration avec les prestataires de soins de santé

- Des rendez-vous de suivi réguliers pour évaluer le traitement et les stratégies de prévention.
- Assurer une communication ouverte et partager ses préoccupations avec l'équipe soignante.
- Se tenir au courant des avancées dans le domaine des déficits immunitaires primitifs.

Résumé

- Des mesures anti-infectieuses doivent être mises en place dès que le déficit immunitaire est diagnostiqué.
- Les patients et leur famille doivent être informés du diagnostic de DIP, de leur sensibilité aux infections et des signes indiquant qu'une consultation médicale est nécessaire.
- Le soignant et/ou le patient doivent comprendre le type et la gravité du déficit.
- Les patients doivent respecter les mesures prophylactiques.
- Maintenir de bonnes pratiques d'hygiène et de soins personnels.
- Signaler rapidement tout signe ou symptôme d'infection.

Nous vous remercions de votre attention.

1. David R. Snydman *et al*, Prevention of Infections During Primary Immunodeficiency, *Clinical Infectious Diseases.* 2014 Nov; 59 (10): 1462-70
2. Papadopoulou-Alataki E, Hassan A, Davies EG. Prevention of infection in children and adolescents with primary immunodeficiency disorders. *Asian Pac J Allergy Immunol*. 2012 Dec; 30(4): 249-58
3. IPOPI Booklet – Primary Immunodeficiency and infections (1st edition)

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La thérapie par immunoglobulines: Du plasma à la médecine

Immunoglobulin therapy: The journey from plasma to medicine

Johan Prevot | Belgium

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Immunoglobulin Therapy: from plasma to medicine

Johan Prévot
Executive Director
IPOPI

COLLABORATION
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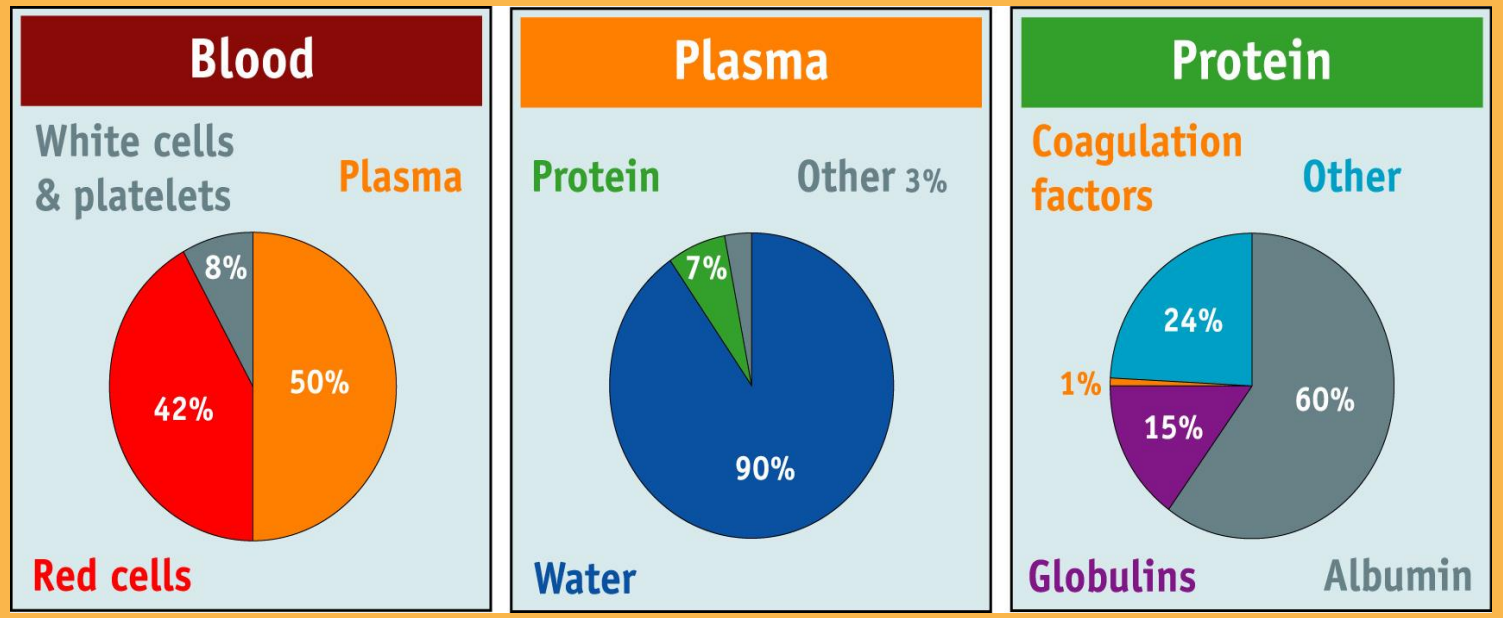
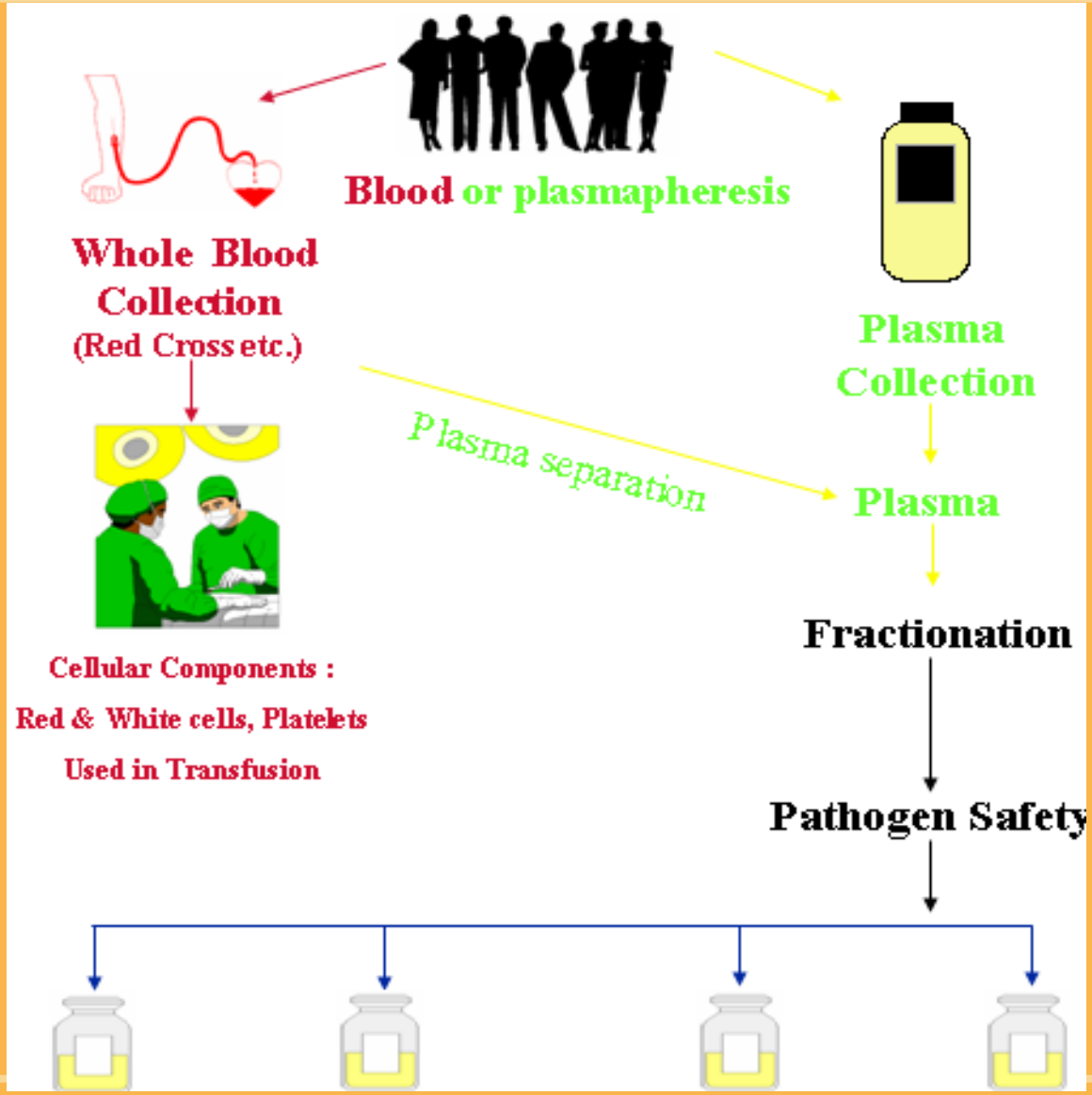
Contents

- Plasma & Blood
- Collection & plasma supply
- Plasma Fractionation
- IG supply
- Global and regional initiatives
- Conclusions

What is plasma?

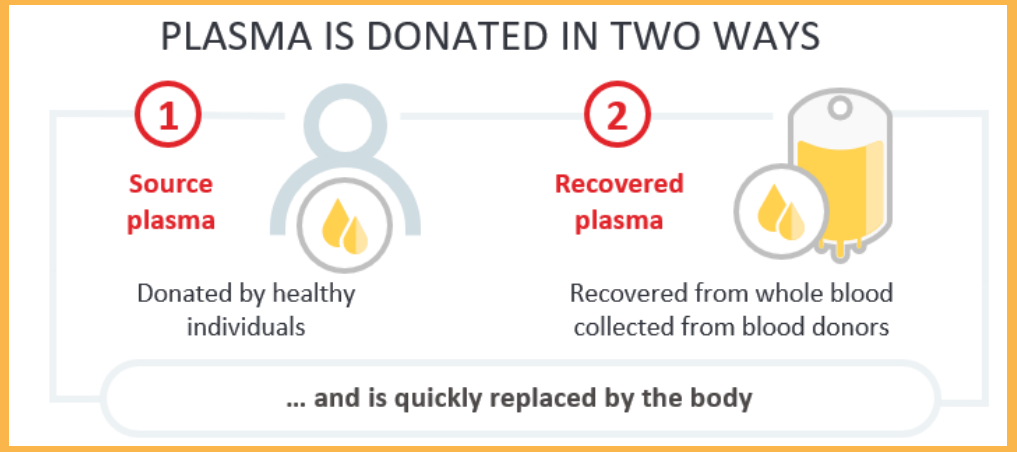


Blood is local, plasma is global



Plasma for the development of IG therapies, needed by a majority of PID patients

- One PID patient needs 130 donations of plasma a year to stay alive



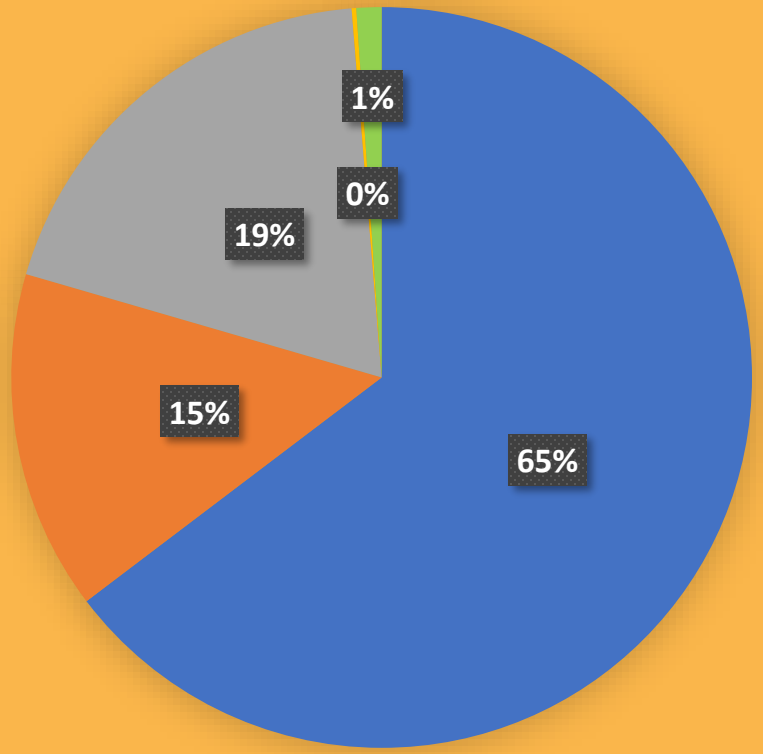
1 liter of plasma
=
3-5 grams of IG

- From whole blood donations (recovered plasma) ~ 200-250ml of plasma per donation. Maximum donation frequency: 4-6 times per year
- Through plasmapheresis (source plasma) – **plasmapheresis allows to collect more (700-800ml), more often as it regenerates in the body within 48 hours**

Plasmapheresis

Longer process than blood donation (ie 1h30)
In a specialized centre

Origin of plasma for fractionation 2020



- North America *
- Europe
- Asia & Pacific **
- Latin America
- Middle East & Africa

Total Plasma Collection volume

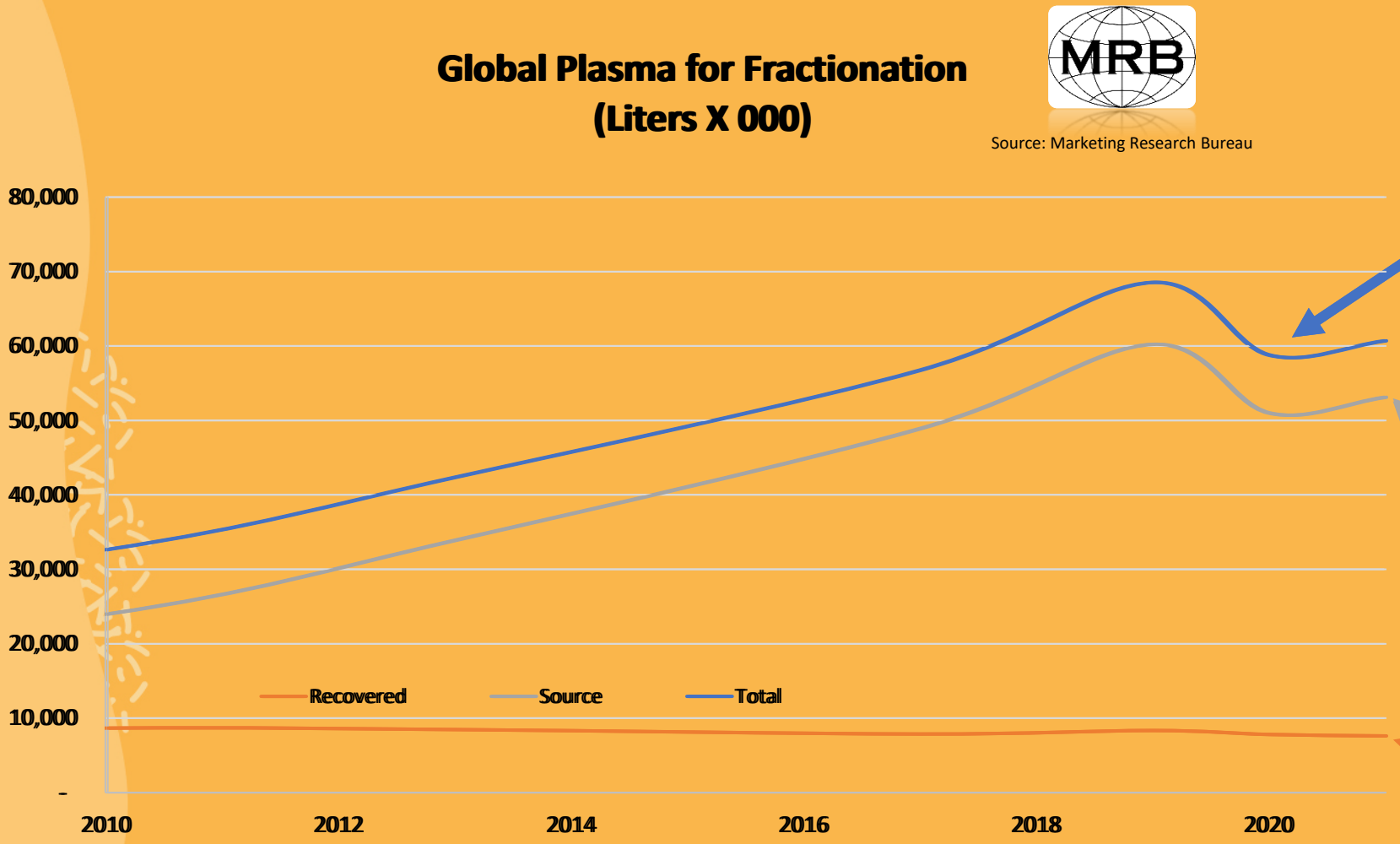
- 2019: 69 M liters
- 2020: 59 M liters (-14% vs. 2019)
- 2021: 62 M liters (-10% vs. 2019)

*United States represented over 99% of the North America total

** China represented almost 75% of Asia & Pacific total

Source: Marketing Research Bureau

Plasma volume trends & impact of COVID19



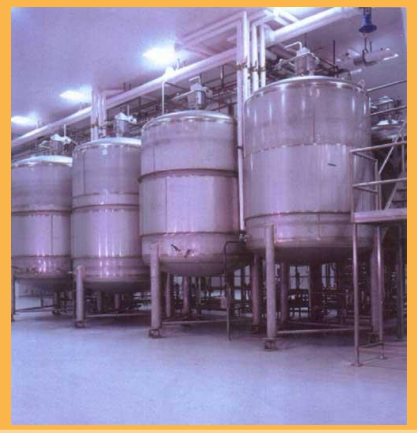
After falling due to the pandemic global plasma volume is increasing again

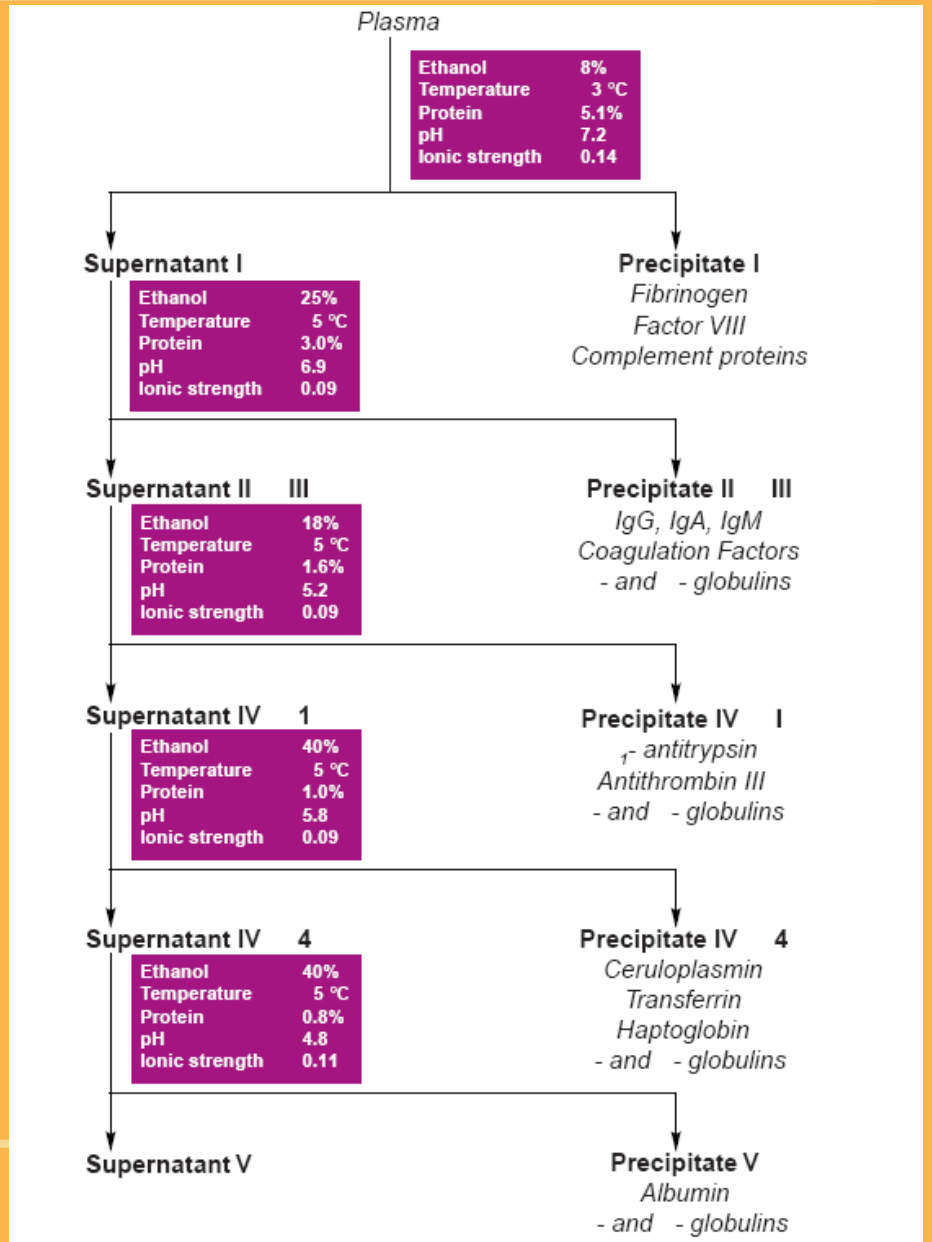
Source plasma was responsible for the growth in 2021. In 2019 source plasma = approx. 90% of the world's plasma

The volume of Recovered plasma (all public) has declined in recent years, including since the COVID-19 pandemic.

Plasma fractionation

- Developed by Edwin Cohn during WW2
- Proteins are purified by adding alcohol to plasma in steel tanks at low temperatures
- The resulting precipitates are further purified into the main therapeutic plasma proteins – albumin, immunoglobulin etc
- Has stood the test of time – relatively little modification in 60 years



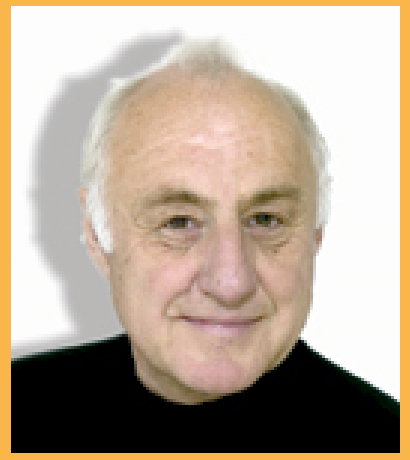


Ethanol fractionation tanks

Protein separation

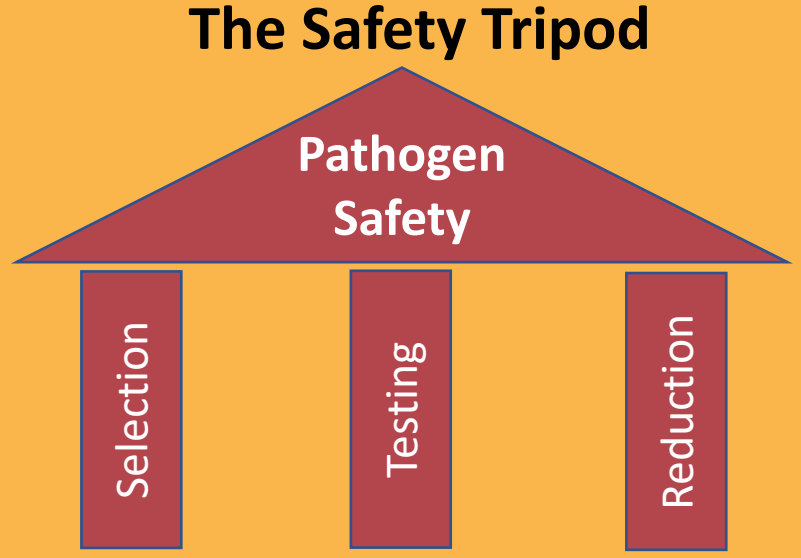
Preparative chromatography

- Developed over past 40 years – pioneered by John Curling in 1970's
- Protein mixtures (eg plasma) are resolved into individual proteins through interaction with a medium (*gel*) which separates them on the basis of size, charge, etc
- Can lead to high purity and biological activity well preserved

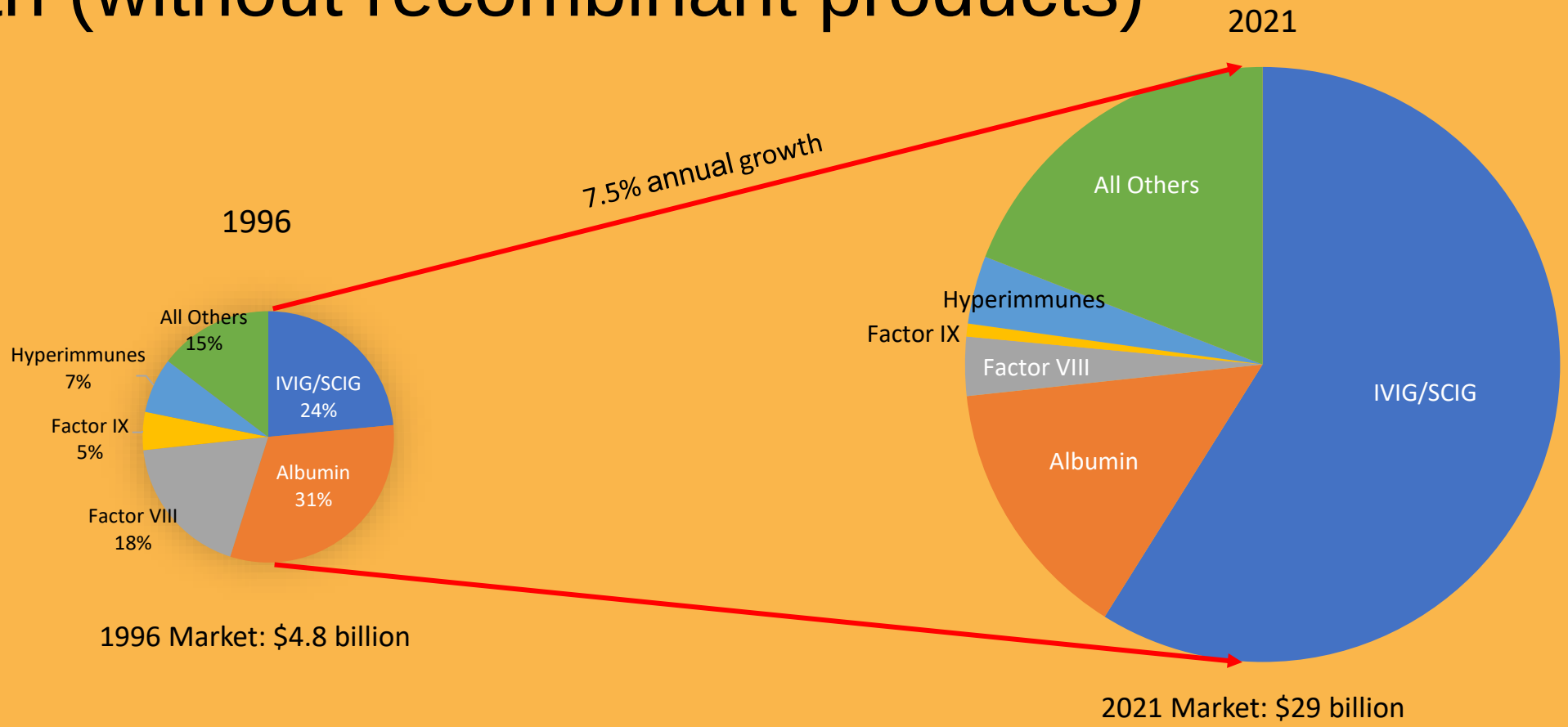


Safety of immunoglobulins

- Biological medicines, zero risk does not exist BUT:
- In well regulated environments, plasma derived medicinal products have demonstrated an excellent viral safety record in the last three decades
  - Robust donor selection procedures & exclusion of at risk donors
  - Robust screening tests
  - **Sophisticated manufacturing process, viral inactivation & removal steps**
- Industry voluntary standards



25 Years of worldwide plasma proteins market growth (without recombinant products)



1996 Market: $4.8 billion

2021 Market: $29 billion

Source: Marketing Research Bureau

Note: Pie charts are drawn to scale

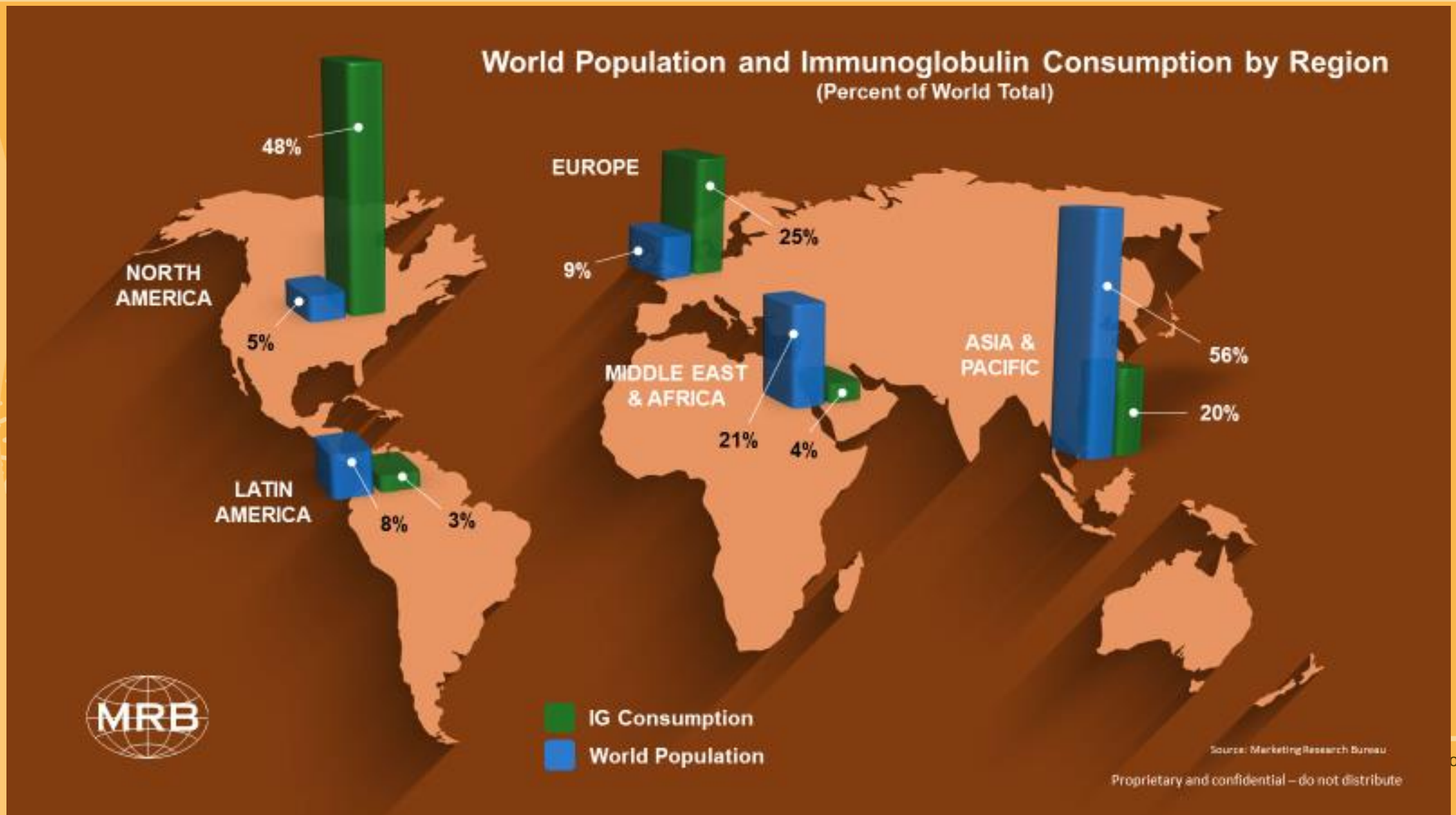
IG usage had grown at a steady rate for the past 12+ years before the pandemic

2021 volume was down 5-6% from 2020 due to plasma collection and IG supply

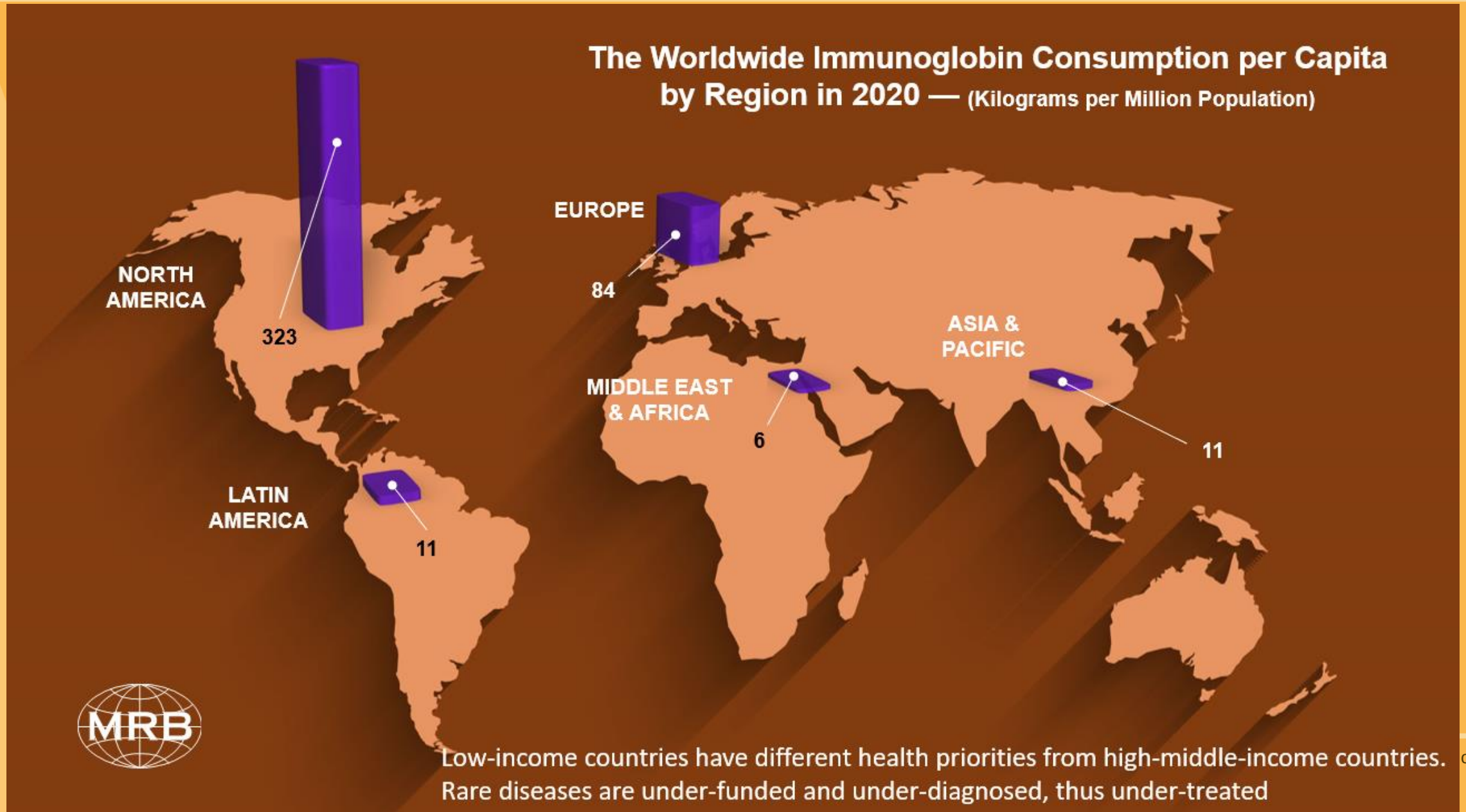
2020:~250 tons sold

Source: Marketing Research Bureau

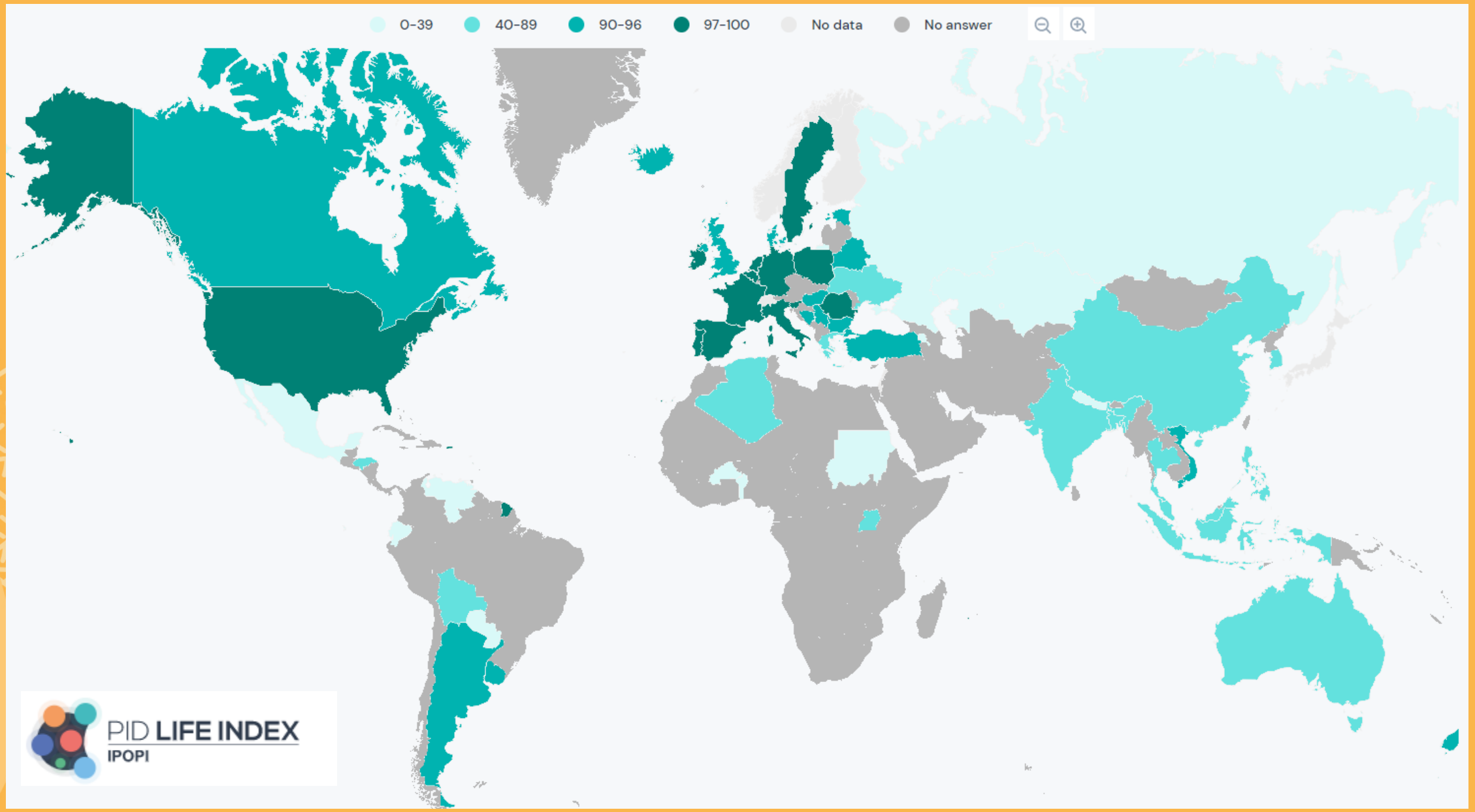
Worldwide IG consumption per region 2020



Worldwide IG consumption per region 2020



IG access globally (all routes)



Global, regional & national initiatives

- Multi-stakeholder joint efforts = key to improving plasma availability
- Need for dialogue around key issues and patient-centred approach
- IG therapies are listed as essential medicines by WHO, they MUST be available, affordable and accessible for patients in need
- Great recent global, regional and national initiatives to push for this:
  - ➢ International Coalition for Safe Plasma Proteins (ICSPP)
    - ✓ WHO, ISBT, IPOPI, WFH, IFBDO, IPFA & PPTA
    - ✓ ICSPP Pilot in Dakar, Senegal - National Blood Transfusion centre
  - ➢ UNITAR "Strengthening healthcare systems to meet patients' needs for PDMPs" with support of Takeda
  - ➢ Grifols Alliance with Egyptian Government

ISBT International Society of Blood Transfusion

About Events Membership Communities Partnerships Working Parties Resources

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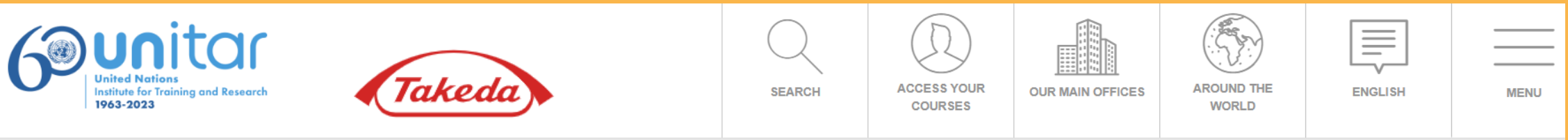
Launch of the International Coalition for Safe Plasma Proteins (ICSPP)

Organizations join with ISBT to advance global access to safe plasma proteins

ICSPP

The ISBT Working Party on Global Blood Safety initiated the ICSPP as a global coalition to advance access to safe plasma proteins in Low- and Middle- Income Countries. In cooperation with the World Health Organization, this coalition was established to address the global insufficiency of plasma-derived medicinal products that are unavailable or unaffordable in many low- and middle- income countries (LMIC) and the consequent suffering and early mortality of patients

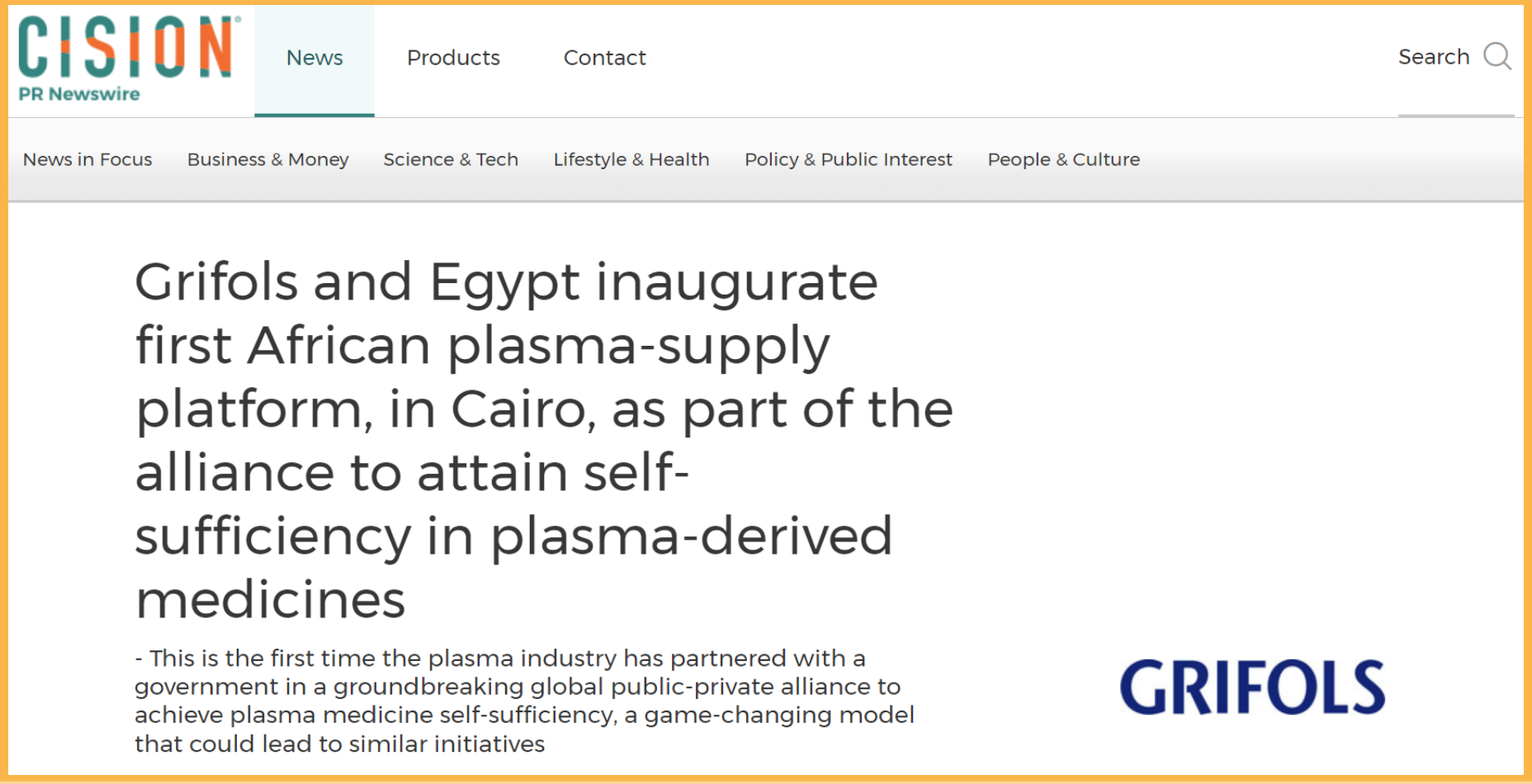


STRENGTHENING HEALTHCARE SYSTEMS TO MEET PATIENTS' NEED FOR PLASMA-DERIVED THERAPIES

29 September 2022, Osaka, Japan, and Geneva, Switzerland – As part of their well-established partnership, the United Nations Institute for Training and Research (UNITAR) and Takeda (TSE:4502/NYSE:TAK), a global pharmaceutical leader and an active member of the United Nations Global Compact, are pleased to announce a new joint initiative focused on **strengthening countries' healthcare systems to meet patients' need for plasma and plasma-derived therapies**. The initiative will draw on UNITAR's expertise in training and education toward the development of innovative solutions to global challenges and Takeda's deep knowledge of rare disease and the plasma ecosystem.

Global demand for plasma has greatly increased over the past 20 years and is continuing to grow due to increasing numbers of patients with rare diseases being diagnosed, as well as higher standards of care and broader access to treatment globally. This demand for life-changing and life-sustaining plasma-derived therapies far exceeds available supply, resulting in more people around the world struggling to access the treatments they need, especially in low- and middle-income countries.

Other initiatives



Conclusions

- Challenges in collecting (GMP quality) plasma and accessing IG therapies remain significant
- African medium IG consumption per capita is lowest in the world
- But:
- Recent international efforts have the potential to significantly improve the situation
  - Plasma collection - By ensuring appropriate policies and strengthening regulatory requirements
  - More plasma, better quality plasma will result into more Igs fractionated for use in the continent
- Increasing collaborations between stakeholders at national and regional level to raise awareness and work at policy level are key
- IPOPI family in Africa is growing - we are by your side!

Thank you!

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Comment les immunoglobulines sont-elles administrées?

How are immunoglobulin therapies administrated?

Houda Chadil | Morocco

IPOPI
8TH AFRICAN
PID PATIENTS'
MEETING
14-15 JUNE 2023
CASABLANCA, MOROCCO
an IPOPI event

HOW ARE IMMUNOGLOBULIN THERAPIES ADMINISTRATED?

HOUDA CHADIL

COLLABORATION
ASID
HAJAR

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PLAN

Immunoglobulins
The different presentations of IVIG (Morocco)
Installing the patient and preparing the medication
Therapeutic premedication
Undesirable effects and their management

IMMUNOGLOBULINS

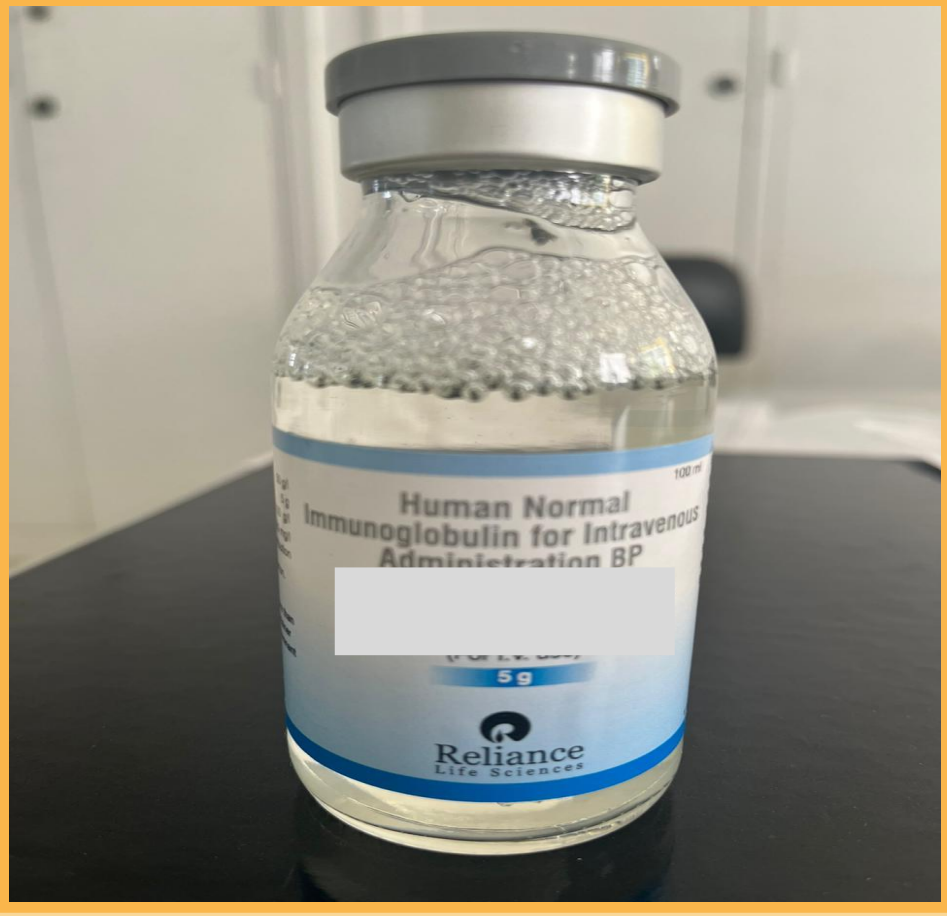
IGIV antibodies are normal antibodies obtained from the plasma of many donors and are also known as venoglobulins.
Indications include :

- Certain immune deficiencies: substitution
- but also in immunomodulation: diseases caused by an abnormal immune reaction: thrombocytopenic purpura, secondary immune deficiency, Guillain-Barré syndrome, Kawasaki disease, etc.

CONTRAINDICATION

Hypersensitivity to human immunoglobulin
Selective IgA deficiency with anti-IgA antibodies
Known hypersensitivity to one of the excipients mentioned in the composition section.

The different presentations of IGIV



INSTALLING THE PATIENT

The patient declares his/her identity (surname, first name, date of birth)
Inform the patient about the treatment
Placement of a peripheral catheter
Hypersensitivity reaction during previous courses of treatment

CHECK LIST

A blood sample is systematically taken between the 3rd and 7th day of each course of treatment to ensure good biological tolerance (IGA, IGG, IGM, ECBC).
Prescriber's agreement to administer the treatment

The medical prescription includes the following information
- Name of the IGIV speciality
- infusion dosage
- duration of infusion
- pre-medication if necessary
- hydration protocol

ADMINISTRATION OF IGIV

-Match between patient identity, speciality, batch number, dose prescribed and treatment expiry date.
-Vials carried at room temperature
-The preparation is labelled with the patient's name and the start time of the infusion.
-Continuous monitoring of the patient: BP, temperature, pain, tolerance.
-Monitoring frequency adapted to the patient.
-Monitoring recorded in the patient's file.

MONITORING

The recommended flow rates are:

0.5 ml /kg/H for the first hour of infusion
1 ml/kg/H for the following half-hour
2 ml/kg/H for the following half-hour
3 ml/kg/H for the rest of the infusion.

- check for signs of inflammation: pain, redness, heat, oedema, etc.

SIDE EFFECTS OF IGIVS

Infusions are generally well tolerated. Possible side effects are usually related to too rapid an infusion, which should be slowed down:

Chills
Hyperthermia
Headache
Nausea
Vomiting
Hypertension
myalgia

PREMEDICATION

Patients with allergic reactions should be premedicated:
Vascular filling
Hydrocortisone hemisuccinate 5 mg/ kg IVD
Prepare hydrocortisone in SAP (auto-push syringe) in parallel with the immunoglobulin infusion.

HOW TO DEAL WITH ADVERSE REACTIONS

Stop the infusion
Reinforce monitoring
Vascular fillingAdminister (analgesics, antipyretics, antihistamines)
Record adverse reactions in the medical record
Secondly: take blood cultures in the event of hyperthermia
Fill in the monitoring sheet, mentioning: type of occurrence, vital parameters, time of occurrence.

THANK YOU

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8TH AFRICAN PID PATIENTS' MEETING
14-15 JUNE 2023
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Q&R

Q&A

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Thérapies curatives: Greffes de moelle osseuse et thérapie génique

Curative therapies: Transplant and gene therapy

Dr. Monia Ouederni | Tunisia

Curative Treatment in Primary Immune Deficiencies
HSCT and Gene therapy

Pr Monia Ouederni

Departement of Pediatrics: Immunopathology, Hematology and Stem Cell transplantation,

National Bone Marrow Center Tunis

Faculty of Medicine, University Tunis El Manar, Tunisia

ASID 2023 Casablanca, IPOPI Session 15 June 2023

PIDs in Africa

PIDs are more common in areas with high rates of consanguineous marriage such as Africa region (20–50%), since most have an autosomic recessive mode of inheritance A significant number of these AR PIDs were first described in patients from Africa

Region/Country	Registered Patients	Year of report	PAD (%)	CID (%)	Phagocytic defects (%)	Complement deficiency (%)	Other PIDs (%)	Genetic diagnosis (%)	Gender ratio M/F	Consanguinity (%)
Tunis	710	2015	17.7	28.6	25.4	0.4	27.9	98 (13.8)	1.4	58.2
Morocco	424	2014	22.7	24.1	15.1	3.1	35.0	22 (5.1)	1.1	43.2
Libya	106	2018	-	-	-	-	-	-	-	-
Algeria	600	2018	-	-	-	-	-	11 (1.8)	-	-
Egypt	476	2016	18.0	29.6	13.2	1.2	37.5	106 (22.2)	1.4	79.7
South Africa	168	2011	50.6	25.0	5.4	4.2	14.8	13 (7.7)	1.5	1.1
Sudan	72	2018	-	-	-	-	-	-	-	-
Total Africa (Maghreb registries)	4509	2018	-	-	-	-	-	-	-	-
Total Africa (JMF registry)	1836	2018	-	-	-	-	-	-	-	-
Total Africa (published registries)	4509	2020	22.0	27.4	17.7	1.6	30.9	250 (5.5)	1.3	54.9

CID, combined immunodeficiency; JMF, Jeffrey Modell Foundation; PAD, primary antibody deficiency; PID, primary immunodeficiency.

1. Abolhassani H, et al. *Expert Rev of Clin Immunol*. 2020;16(7):717–732; 2. Bousfiha AA, *Tunis Med.* 2018;96(10–11):672-677; 3. Galal N, et al. *J Clin Immunol*. 2016;36:649–655; 4. Erjaee A, et al. *S Afr Med J*. 2019;109:3–11; 5. Mellouli F, et al. *J Clin Immunol.* 2015;35:745–753; 6. Modell V, et al. *Immunol Res*. 2018;66:367–380.

How to treat primary immune deficiencies?

- Diagnosis, principles of care and access to treatment varies widely between different regions of the world and even from country to country within the same continent
- PIDs treatment aim to improve patients' quality of life and reduce the risk of severe infections.
- Over half of the patients have antibody deficiencies, their treatment consists of replacing the missing antibodies
- Many PIDs could be chronic life-long, serious, and even fatal in absence of a curative treatment correcting the underlying dysfunction of the immune system .
- Recent advancements in medical research have paved the way for potential curative interventions.

PID Management in Africa

- Patient outcomes are largely determined **by early recognition and referral** to a centre with appropriate expertise in the management of PIDs. PID management includes:

✓ **Antimicrobial prevention**	✓ **Diagnosis and treatment of occult infections**	✓ **Intravenous IgRT** for antibody deficiencies and CID	✓ **Haematopoietic stem cell transplantation (HSCT)**	✓ **Adjuvant treatments depending on the type of PID:** immunosuppressors, GCSF, IFN-γ

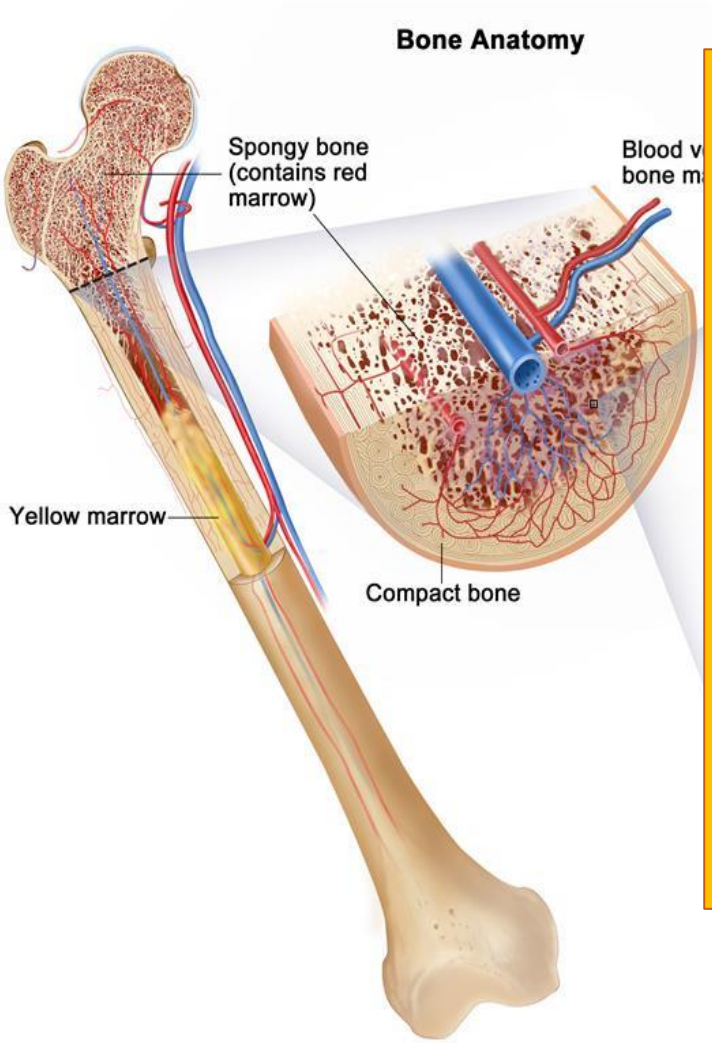
- **Other therapies less/not available in Africa:**
  - **Enzyme therapy** (Recombinant ADA enzyme)
  - **Targeted therapies** for some PIDs e.g.,: CTLA-4-Fc fusion protein (Abatacept, Belatacept), PI3K inhibition, JAK inhibition (STAT1-GOF), Anti-CD20, IL-6R inhibition, Cytokine (IL-1, TNF) inhibitors, C5 inhibitor, Plerixafor (CXCR4 antagonist)
  - **Gene therapy**
  - **Thymus transplantation**

ADA, adenosine deaminase; CID, combined immunodeficiency; CTLA, cytotoxic T lymphocyte antigen; IFN-γ, interferon gamma; IgRT, immunoglobulin replacement therapy; JAK, janus kinase inhibitor; GCSF, Granulocyte colony-stimulating factor; IL, interleukin; MEA, Middle East and Africa; PID, primary immunodeficiency; PI3K, phosphoinositide 3-kinase; STAT1-GOF, signal transducer and activator of transcription 1 gain of function; TNF, tumour necrosis factor.

WHAT IS HAEMATOPOIETIC STEM CELL TRANSPLANTATION?

- HSCT or bone marrow transplantation is a potential curative option for many types of PID
- This procedure was first performed in 1968 on a 22-month-old male infant suffering from SCID
- BMT aims to replace the faulty immune system with an immune system from a healthy donor.
- The stem cells collected from a healthy donor and given to a patient with PID will take up residence in the recipient’ s bone marrow and start to generate healthy immune cells capable of fighting infections.

What are stem cells?



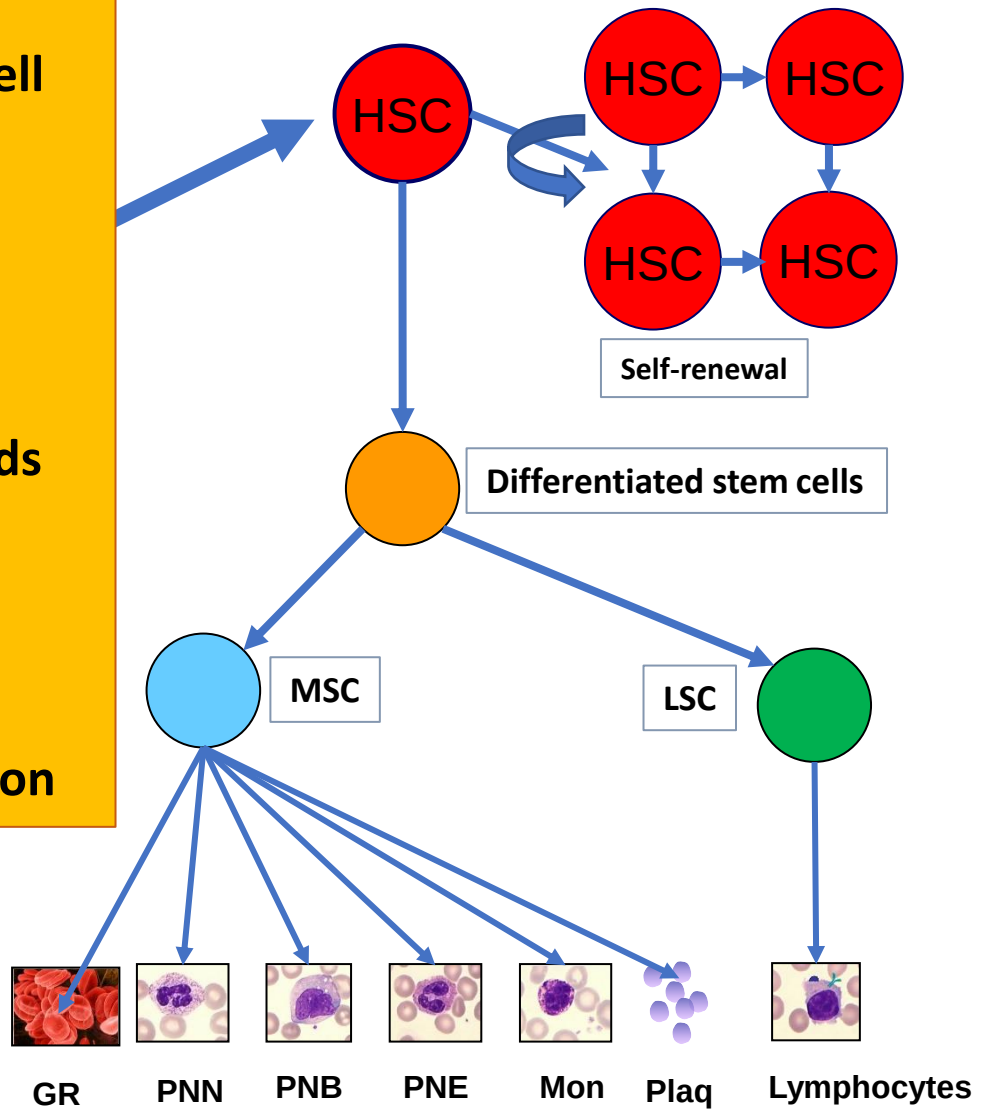
Hematopoetic stem cell

CD34+

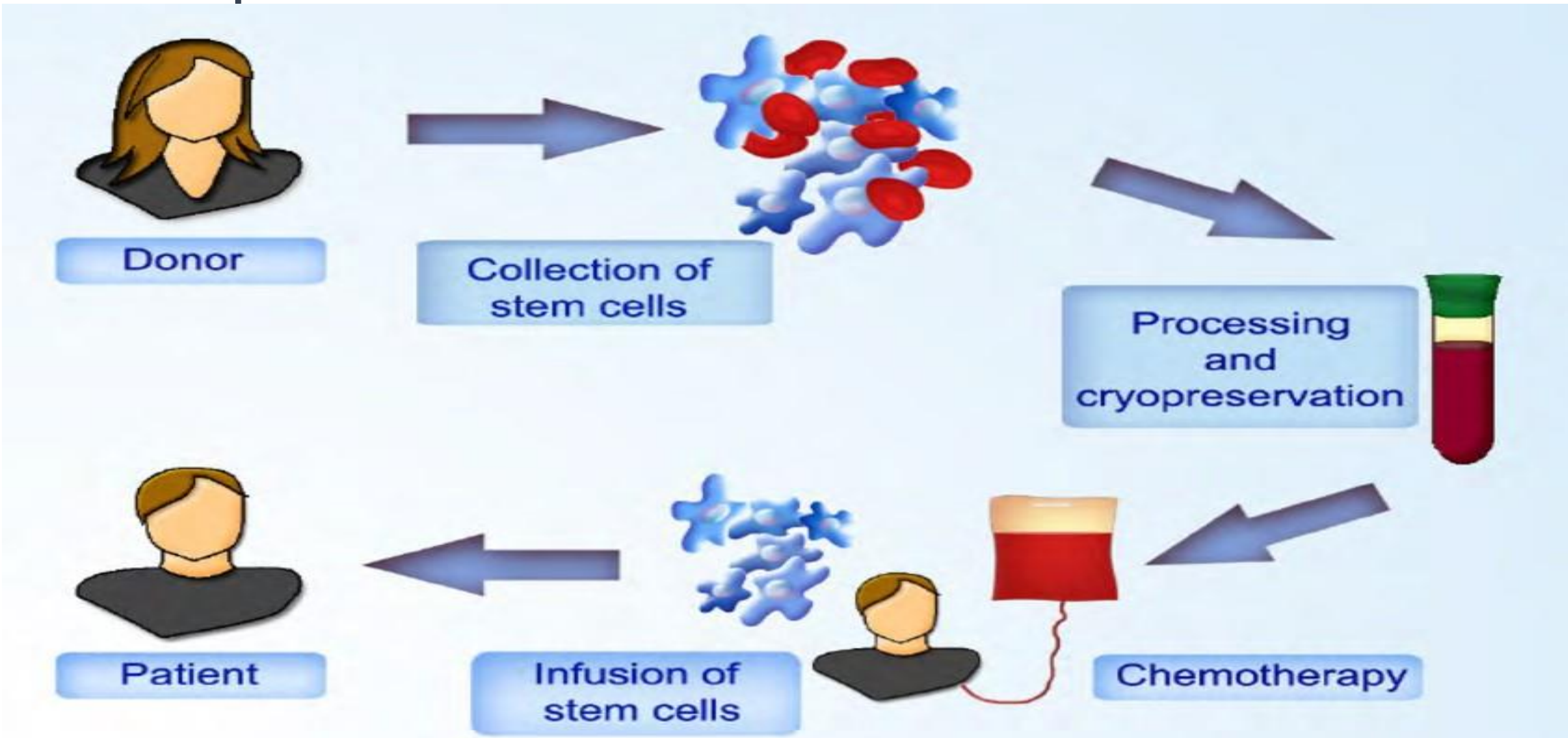
Self-renewal

Differentiation towards blood lines

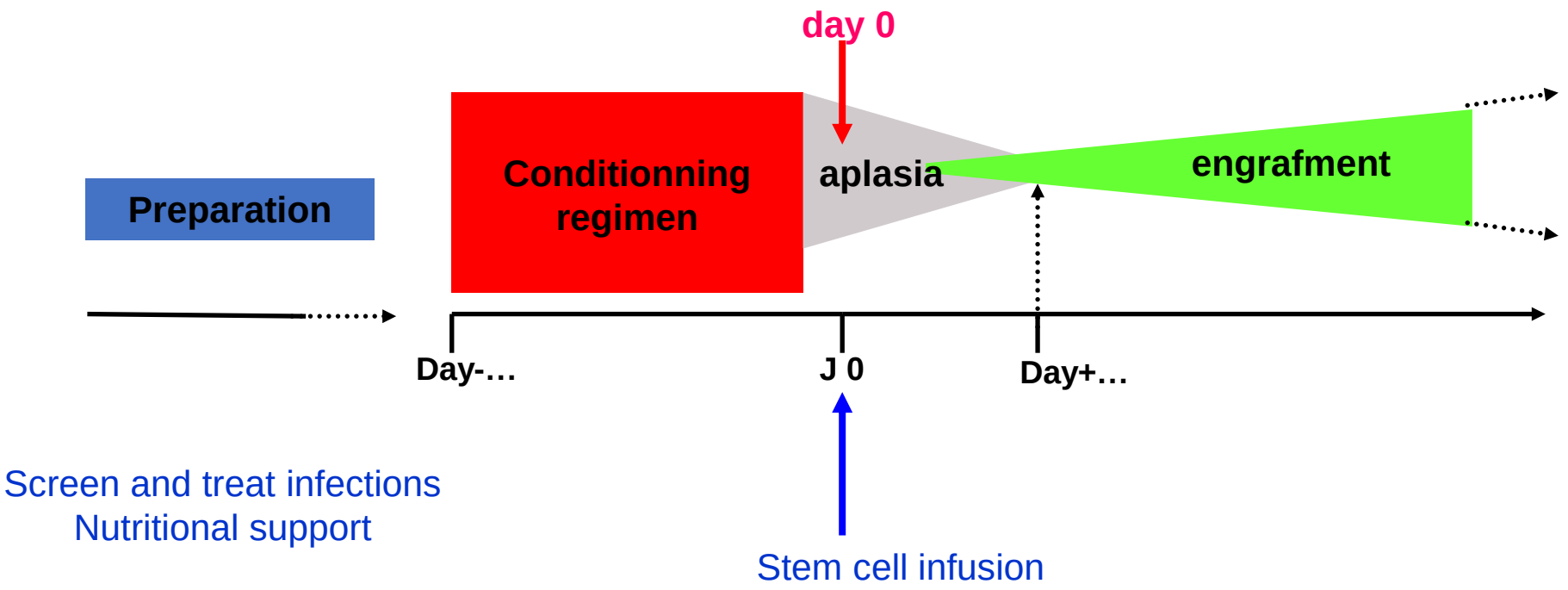
Hematopoietic reconstitution after chemotherapy/radiation



How to perform HSCT ?



Steps of HSCT?



Conditioning regimen is a preparatory treatment aiming to optimize engraftment and reduce the risk of graft rejection or graft-versus-host disease (GVHD)

How to choose the more suitable donor?

HLA-typing:

by high resolution, allele level HLA-A, -B, -C and -DRB1, -DQB1 typing is mandatory for the patient and the donor

Matched sibling donor (MSD)	allele-level matching at all 10 loci is the gold standard	<30% patients have MRD available, risk of disease-carrier status
Unrelated donor	**MUD,** allele-level matching at all 10 loci . **MMUD:** allele-level matching at ≤9/10 loci . Some centers would consider a 9/10 as a MUD	1 in 1 million**,** time spent in waiting for a suitable donor and/or **n**o access to the UDs Registries
Haploidentical family donor	**MMRD:** immediate available familial donor	Higher rate of viral infections, high laboratory expertise required, risk of disease-carrier status

- Matched Unrelated donors **(MUD),**
- Mismatched unrelated donor **(MMUD)**

- **Cord blood units are less used in PIDs because of l**onger immune reconstitution, limited amount of available CD34+ cells, high rate of viral infections, unable to go back to donor for more cells

2. Who is suitable for HSCT?

Factor1 to consider: The type of PIDs

HSCT is Curative	HSCT partially curative	HSCT controversial
SCID, CID*, CGD	Cartilage Hair Hypoplasia	CVID
DOCK8 deficiency, DOCK2 deficiency	PGM3 deficiency	Agammaglobulinemia
IPEX, WAS, WIP deficiency, ARPC1B deficiency,	STAT1-GOF	Complement deficiencies (other than C1q deficiency)
CD40 ligand, CD40 deficiency	STAT3- GOF	DGS
XLP1, XLP2	Severe congenital neutropenia	IKBA deficiency
APDS	ADA2 deficiency	NEMO deficiency
MHC Class II deficiency	CIQ deficiency	
AD Hyper IgE syndrome	CD25 deficiency	
CTLA4 , LRBA deficiency	IL-10 deficiency	
Familial HLH types 1–5	IL-10 Receptor deficiency	
GATA2 deficiency, RAB27A deficiency	DNA double-strand	
LAD I, Reticular Dysgenesis	break repair disorders	

Clinical and immunologic diagnosis of PID

PID diagnosis

ESID CRITERIA

PID classification

IUIS Classification

PID needing HSCT

1. CDI with associated or syndromic features
2. Combined immunodeficiency (CID)
3. Diseases of immune dysregulation
4. Defects in intrinsic and innate immunity
5. Congenital defects of phagocyte number, function, or both
6. Predominantly antibody deficiencies
7. Auto-inflammatory disorders
8. Complement deficiencies
9. Phenocopies of PID

2. Who is suitable for HSCT?

Factor 2 to consider: the clinical status of the patient

1. **Infectious status:**

- For severe PIDs with early onset such as SCID: HSCT is an emergency within the first few months of life before major infections occur

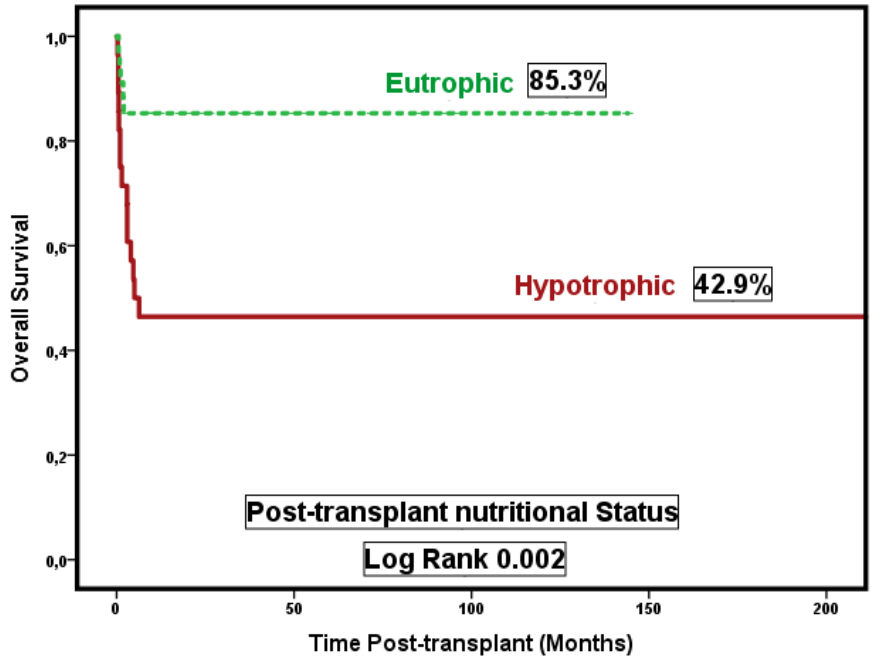
- An ongoing infection should be treated before HSCT to increase the chance of success

2. **Organ damage:** especially for older children or adults, the best outcomes are for those with limited damage to organs such as the liver, heart and lungs from the infections associated with PIDs

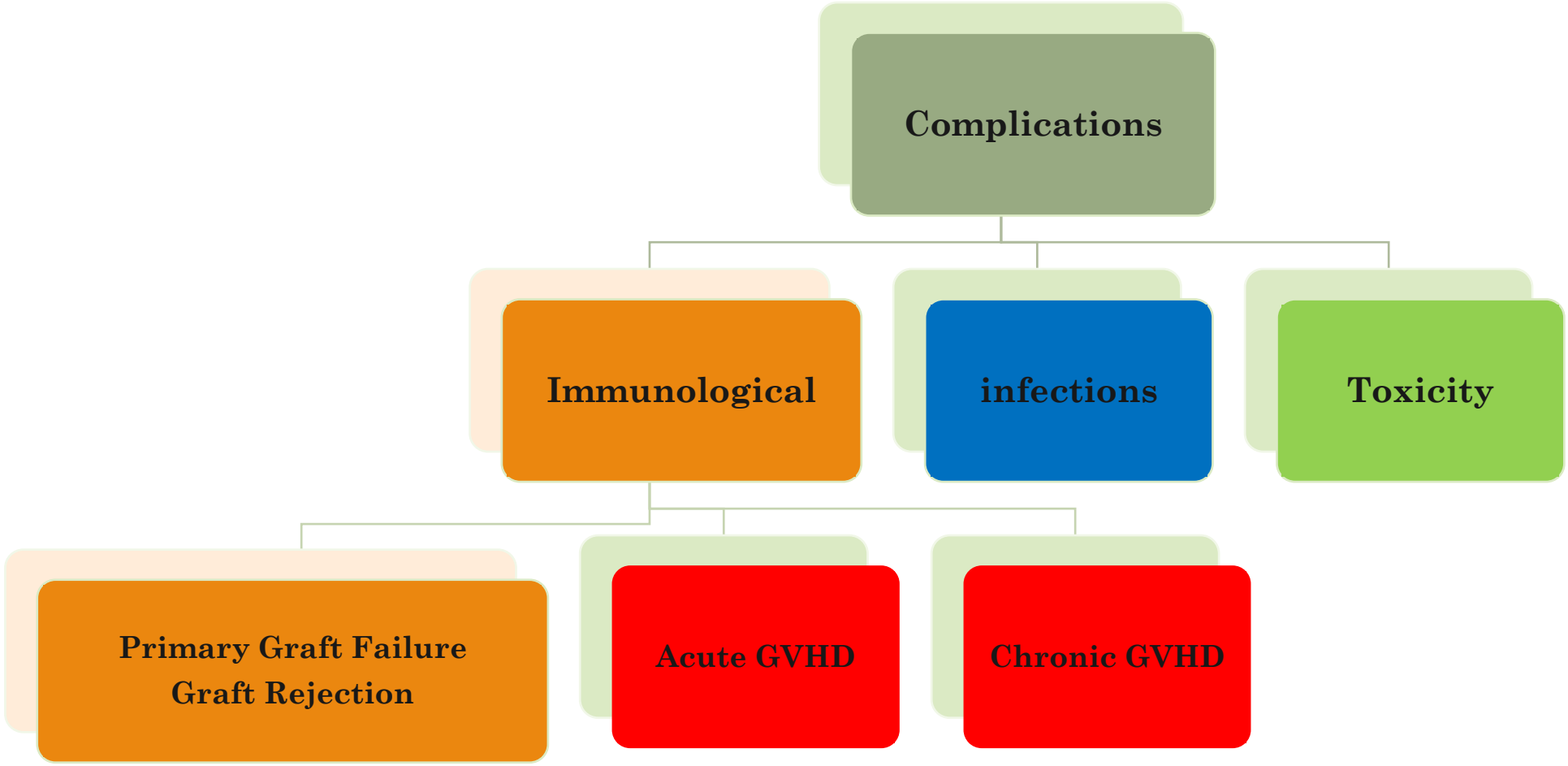
3. **Nutrionnal status**

Table 4. Results of Multivariate Analysis of Outcomes and Contributing Factors.

Outcome and Contributing Factors	Percent with Outcome (95% CI)	Relative Effect (Hazard Ratio for Death)	P Value
Survival at 5 yr			
Age at transplantation and infection status			<0.001
0–3.5 mo	94 (85–98)	1.00	
>3.5 mo, active infection	50 (39–61)	10.88	<0.001
>3.5 mo, infection resolved	82 (70–90)	2.88	0.07
>3.5 mo, no infection	90 (67–98)	1.03	0.97



WHAT ARE THE RISKS ASSOCIATED WITH HSCT?



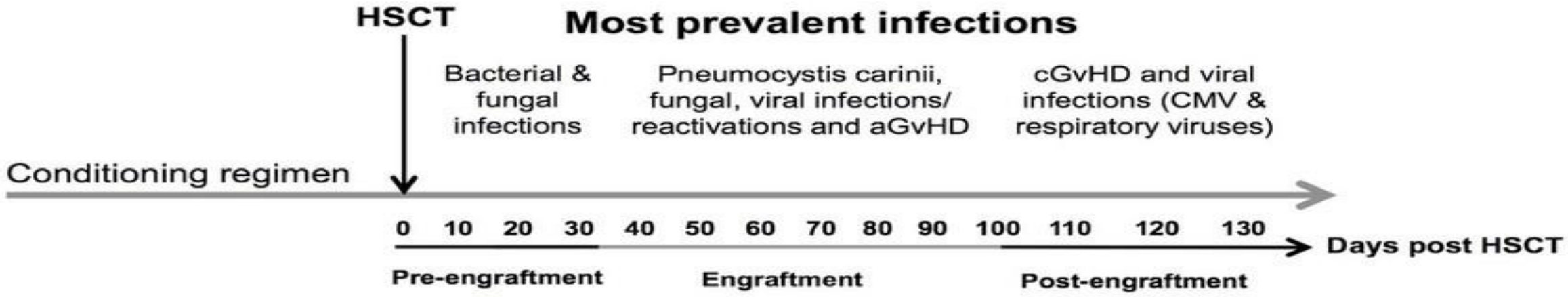
WHAT ARE THE RISKS ASSOCIATED WITH HSCT?

1. **Graft-versus-host disease (GvHD):** an interaction between donor's reactive T-cells and host epithelial cells of Skin, Liver, bowels, Lung, eyes..GvHD prophylaxis is based on immunosupressors.

2. Graft failure : A lack of donor HSC engraftment after HSCT, or a Graft rejection caused by immune rejection of donor cells mediated by host cells

3. Toxicity: Mucosa, Gut, heart, CNS, Liver, kidney, bladder…

4. Infections: A great risk because immune reconstitution can take up to 6 months, prophylactic antibiotics and immunoglobulins could be needed

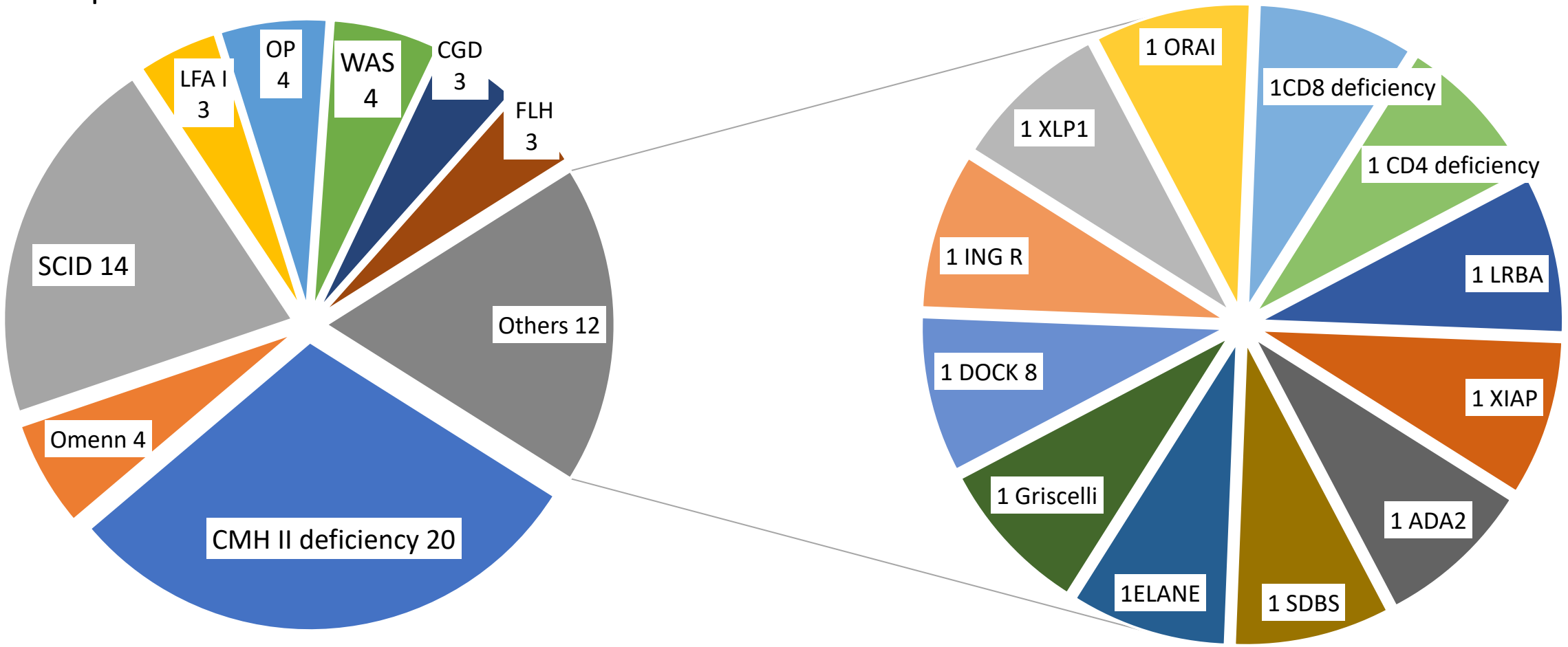


The main centres performing HSCT for PID in Africa are in Tunisia, Egypt, followed by fewer HSCTs for PIDs in other countries such as Algeria, South Africa, Morroco, Kenya…

HSCT experience of one center from Africa

- **The Department of paediatrics: immuno-hematology and stem cell transplantation in National Bone Marrow Transplant Centre, Tunis, Tunisia, is the national referral centre for primary immunodeficiency care, *Created since 1998***
- Patients are referred by physicians and especially paediatricians from all regions of Tunisia for **diagnosis and management** of Primary immunodeficiencies
- We receive also, **primary immunodeficiency** patients from the Maghreb and Africa for diagnosis and treatment

HSCT are done for various primary immune deficiencies in the Pediatric Department

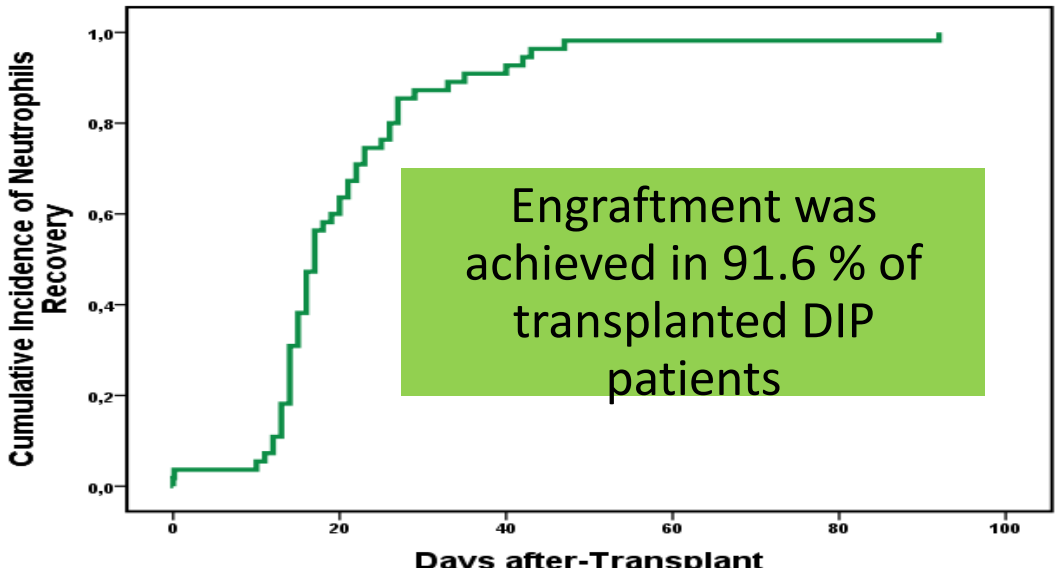


Unpublished data of Dr Ouederni.

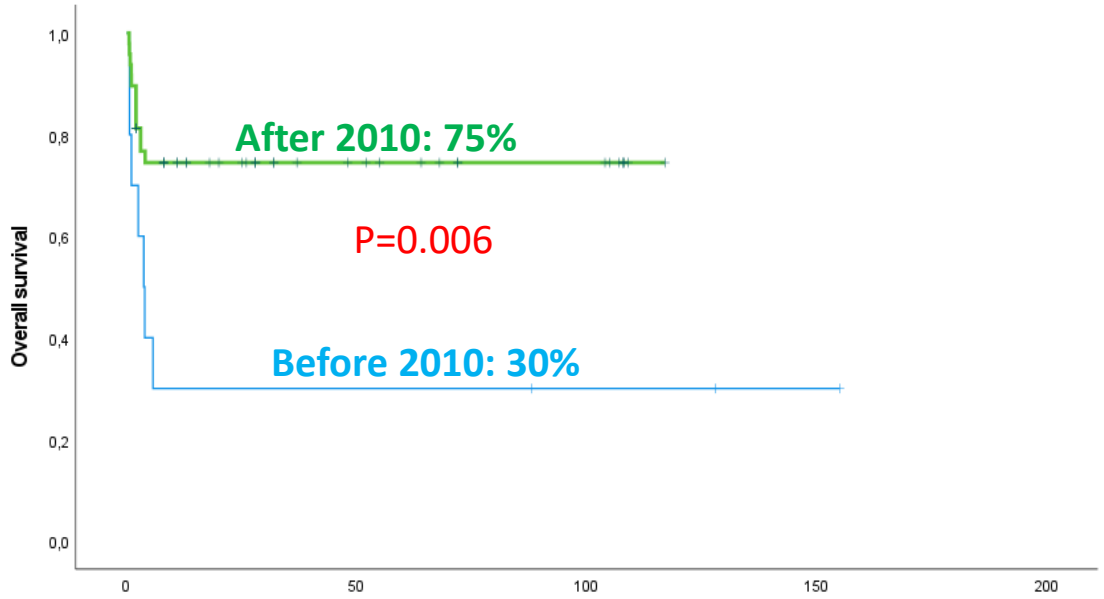
ADA, Adenosine deaminase 2; CGD, Chronic granulomatosis disease; DOCK8, Dedicator of cytokinesis 8; ELANE, neutrophil elastase; FLH, Familial lymphohistiocytosis; ING-R, Interferon gamma receptor deficiency; SCID, severe combined immunodeficiency disease; CGD, chronic granulomatosis disease; LFA-1, lymphocyte function-associated antigen1; LRBA, lipopolysaccharide (LPS)-responsive and beige-like anchor protein; OP, Osteopetrosis; SCN, severe congenital neutropenia; SBDS, Shwachman-Bodian-Diamond syndrome; WAS, Wiskott Aldrich Syndrome; XIAP, X-linked inhibitor of apoptosis protein; XLP1, X-linked lymphoproliferative disease type 1.

Ouederni M, et al. Presented at ESID 2022. Poster 1084.

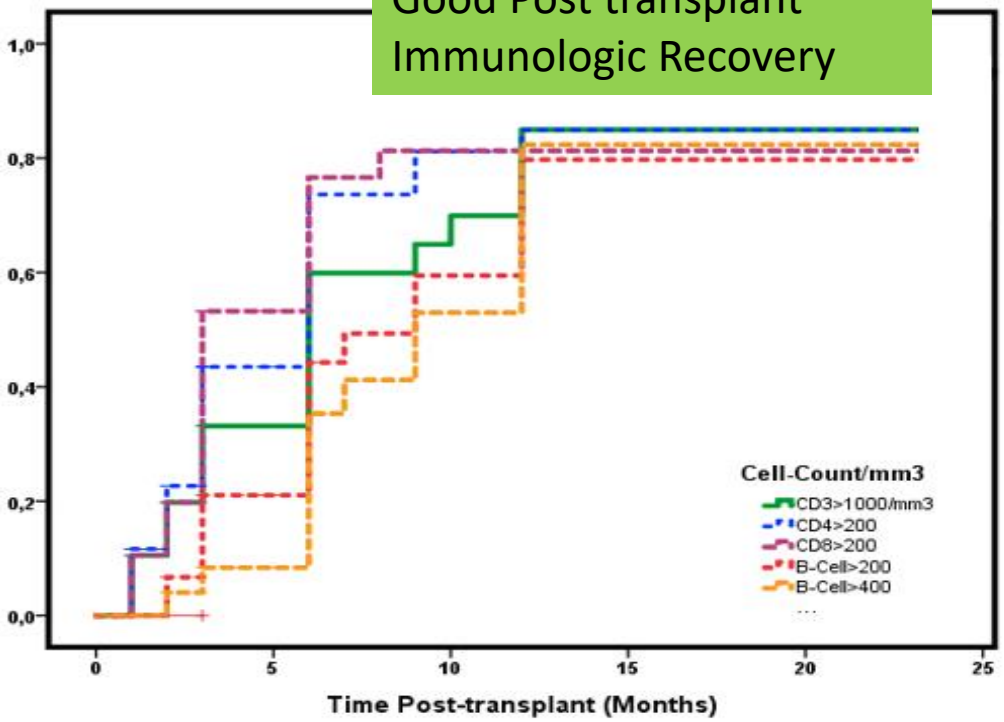
Results of HSCT in PIDs: (exemple of Tunisia)



Engraftment was achieved in 91.6 % of transplanted DIP patients



Good Post transplant Immunologic Recovery



Improved overall survival of Transplanted DIP patients during the last 10 years with less toxic based conditionning regimen

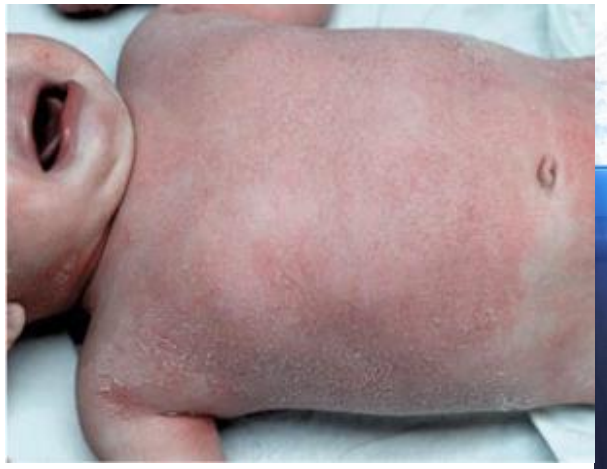
What to do if no sibling mached donor available?

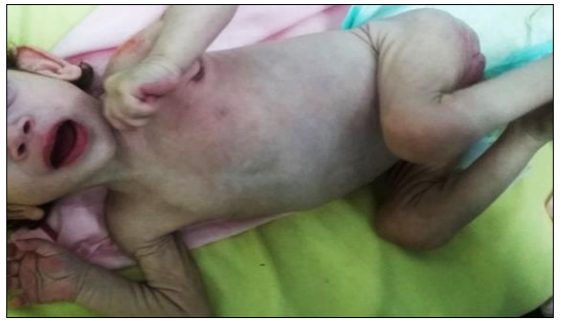
- In the Tunisian context from 1998 to 2015 **the only feasible HSCT is from MRD**
- There is no access to the International Network of UDs Registries, neither to UCB units
- Very high cost for the state if patient is referred to HSCT outside of Tunisia
- Long time spent in waiting for a suitable donor to be identified through donor registries
- In addition, the ex vivo T cell depletion is not available in Tunisia because of the high cost involved in graft manipulation
- Hence, in the context of urgent need to transplant patients that didn’t have identical sibling, we introduce the new activity of T replete Haploidentical HSCT in the department of pediatrics, bone marrow transplant center in 2015

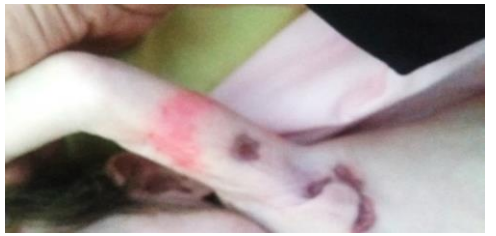
The case of Farah

- Farah born on 12/12/2014 from consanguineous parents.
- A brother who died at 8 months by SCID T-B-NK+ (BCGitis)
- NO BCG at birth
- SCID T-B-NK+
- Double heterozygote mutations for RAG1 gene:
- Now sibling donor was available,
- Haploidentical HSCT from her mother was done at the age of 3 months on 14 March 2015

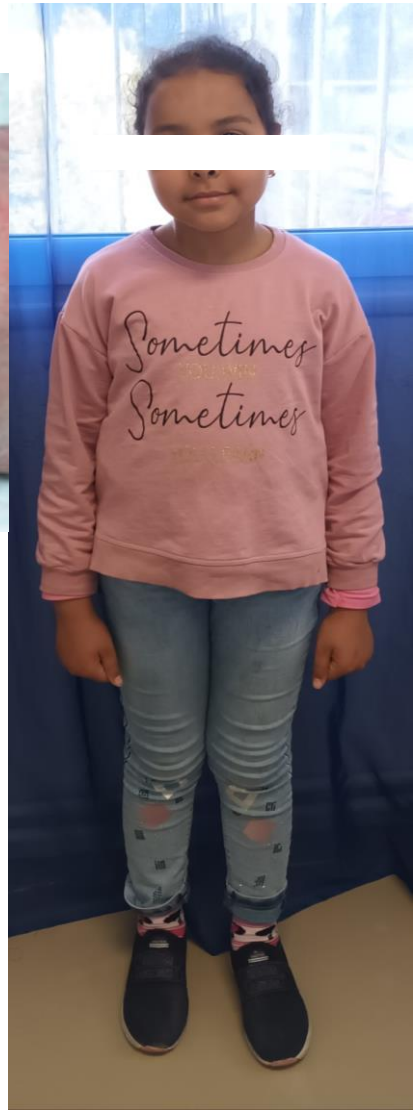
- She is now 7-year-old (transplanted)
- Molecular chimerism: 100% donor
- Event and infection free, IVIg replacement stopped one year after HSCT
- Normal lymphocyte subtypes levels
- She goes to school with good quality of life







Brother



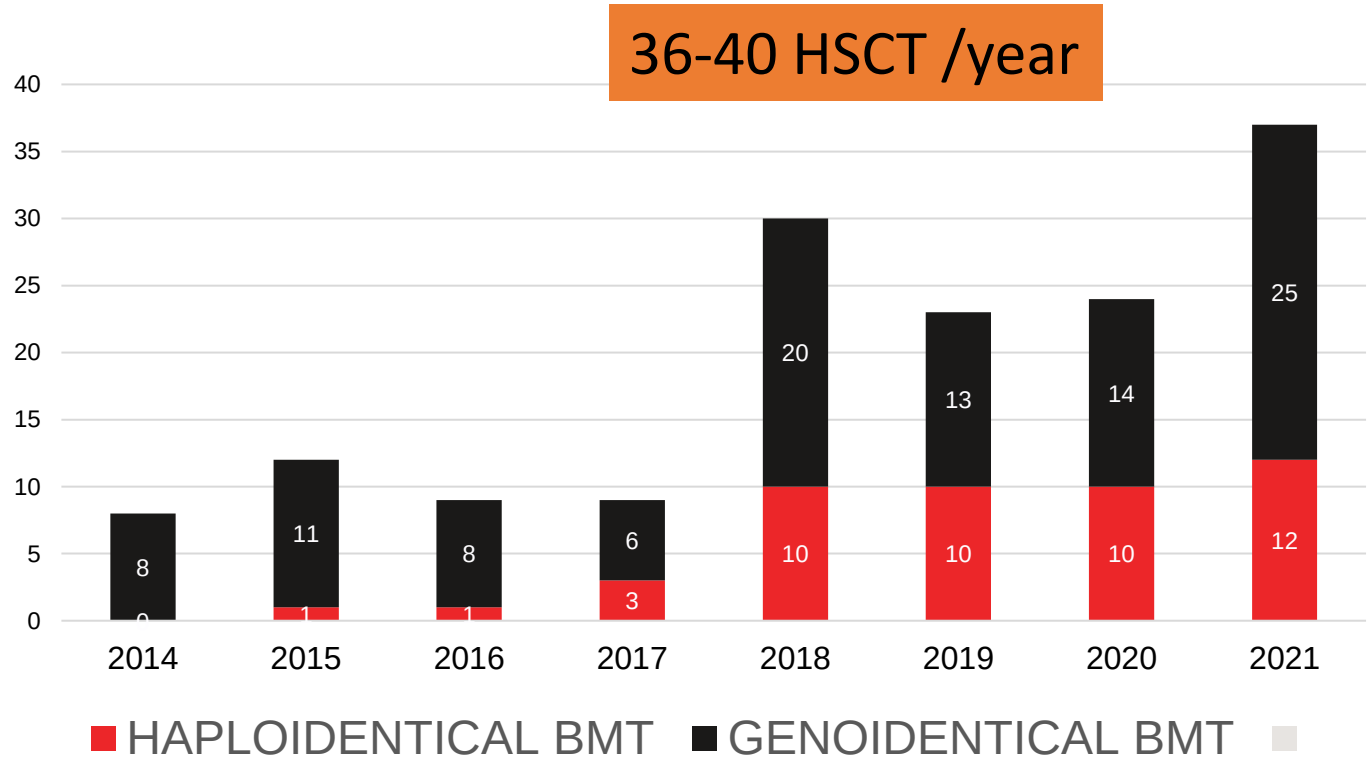
Farah

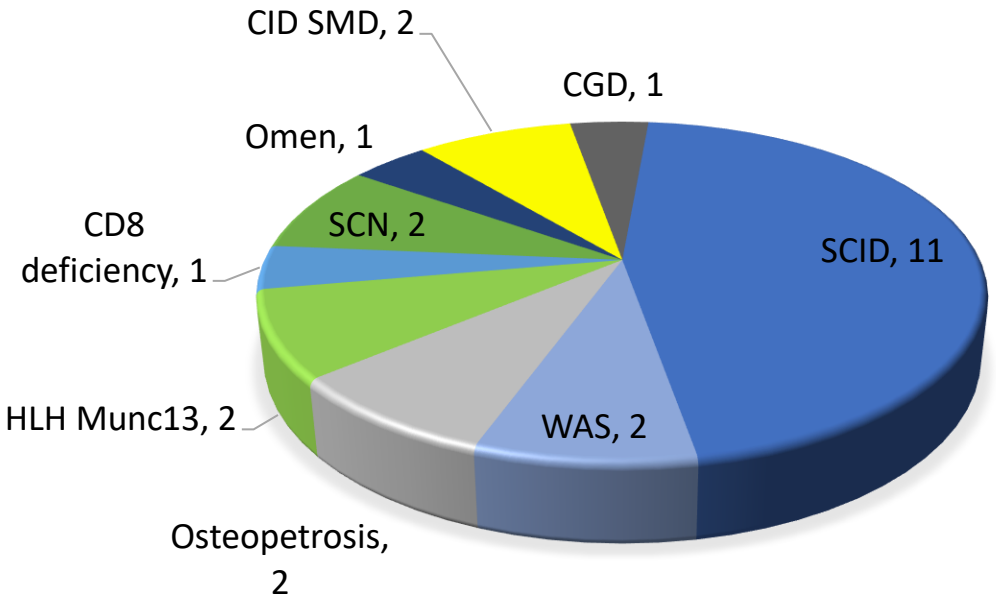
OUEDERNI M et al , Successful Haploidentical T replete HSCT in SCID: First report, journal of clinical immunology, 2016

Regular increase in paediatric bone marrow haploidentical transplant activity

PIDs receiving Haplo HSCT

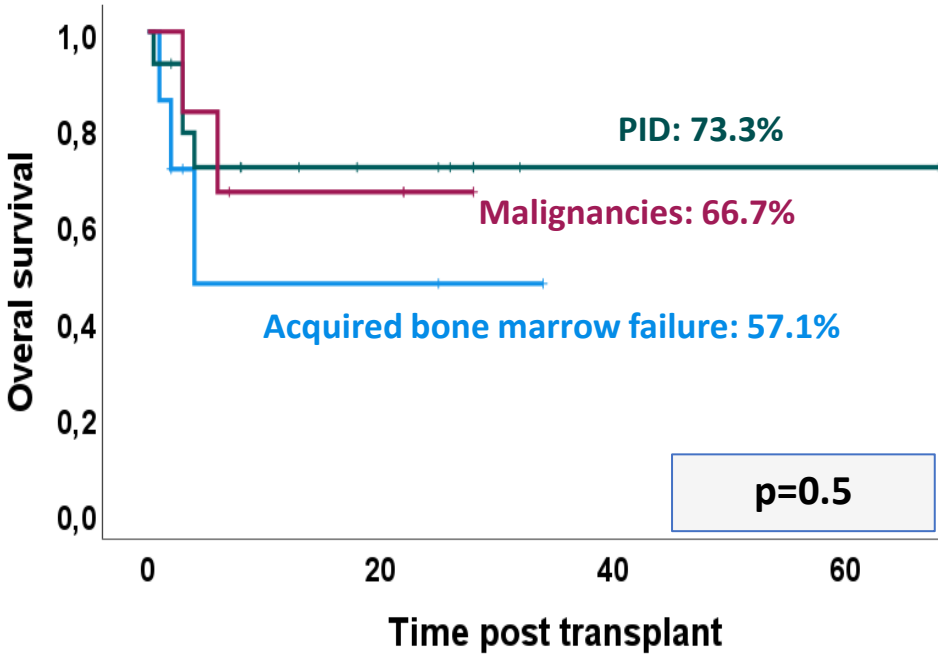
- Half of the patients were **diagnosed before 6 months of age**
- Half of the patients were aged less then **11 months at HSCT**

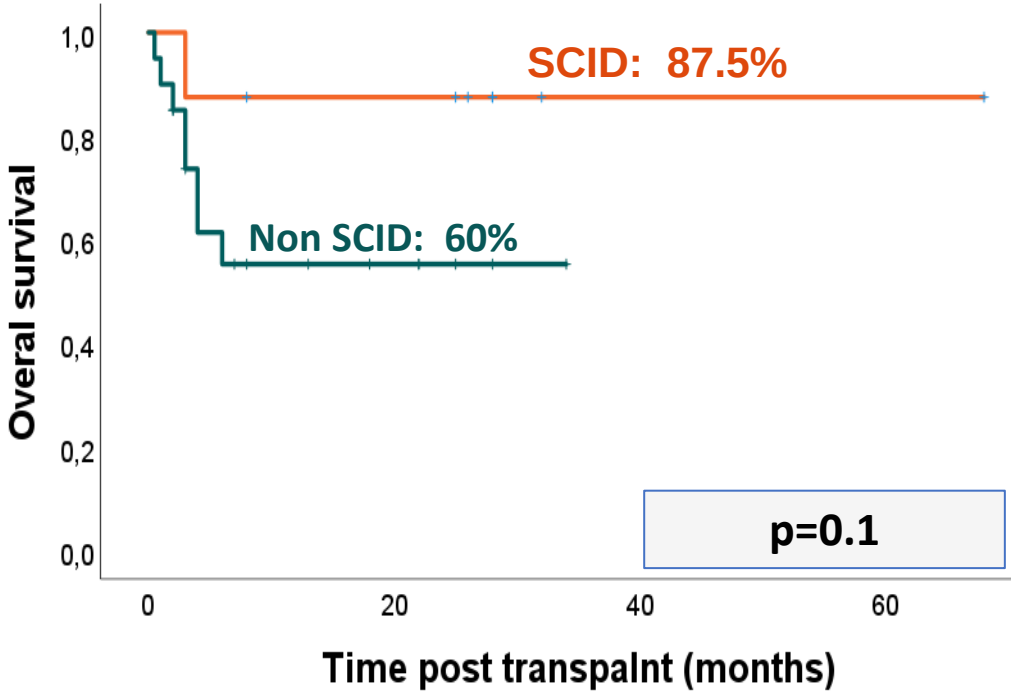




PIDs and especially SCID patients have the best overall survival after Haploidentical HSCT with *in vivo* T depletion

- After a median follow-up of 20 months (3 months—7 years), 75% of patients are alive and 68 % have been completely cured after Haploidentical HSCT





Unpublished data of Dr Ouederni.

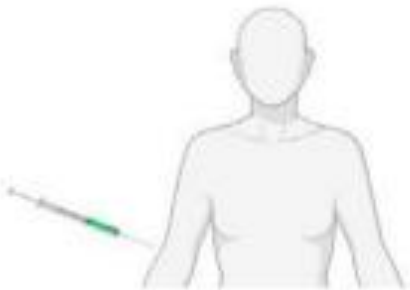
HSCT: long term follow-up

- **PIDs could be completely cured by HSCT.**
- However, this is an intensive and prolonged intervention that requires extended hospital stays for weeks or months and then requires long term outside follow-up to screen sides effects
- The role of a network of family and friends, and also patient's organizations is crucial
- BMT Tunisian association, Joie de vivre , Maram ..are national patient associations having a major role in supporting these patients especially those who need to travel outside their home or country to receive treatments as they are likely to be away for weeks or even months

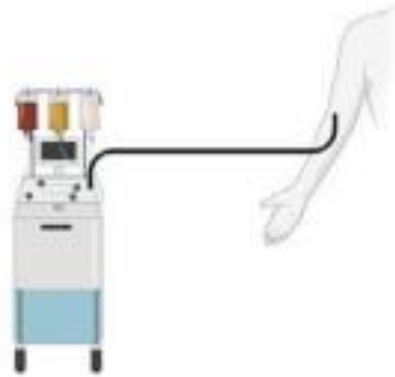
What is Gene therapy?

- Gene therapy aims to correct genetic defects by introducing functional genes or modifying existing genes.
- The process involves three main steps:
  - ✓ delivery of therapeutic genes,
  - ✓ integration into the patient's cells,
  - ✓ and expression of functional proteins.
- Different approaches including viral vectors (modified viruses that can efficiently deliver therapeutic genes to target cells), non-viral vectors, and genome editing techniques.

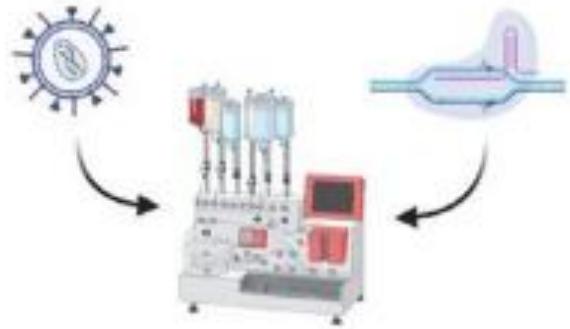
1 Administer treatment to the patient to release CD34+ stem cells from bone marrow into the bloodstream



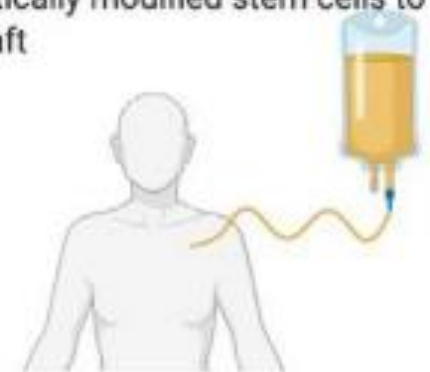
2 Collect the CD34+ stem cells from the bloodstream



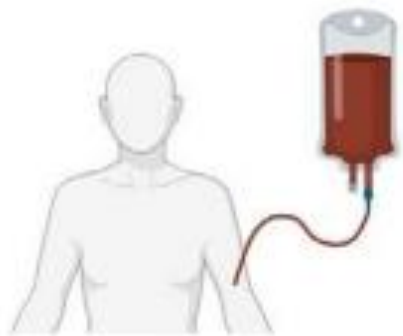
3 Genetically modify the CD34+ cells using viral vectors or gene editing techniques to correct the genetic defect



4 Administer conditioning to deplete existing stem cells and enable the genetically modified stem cells to engraft



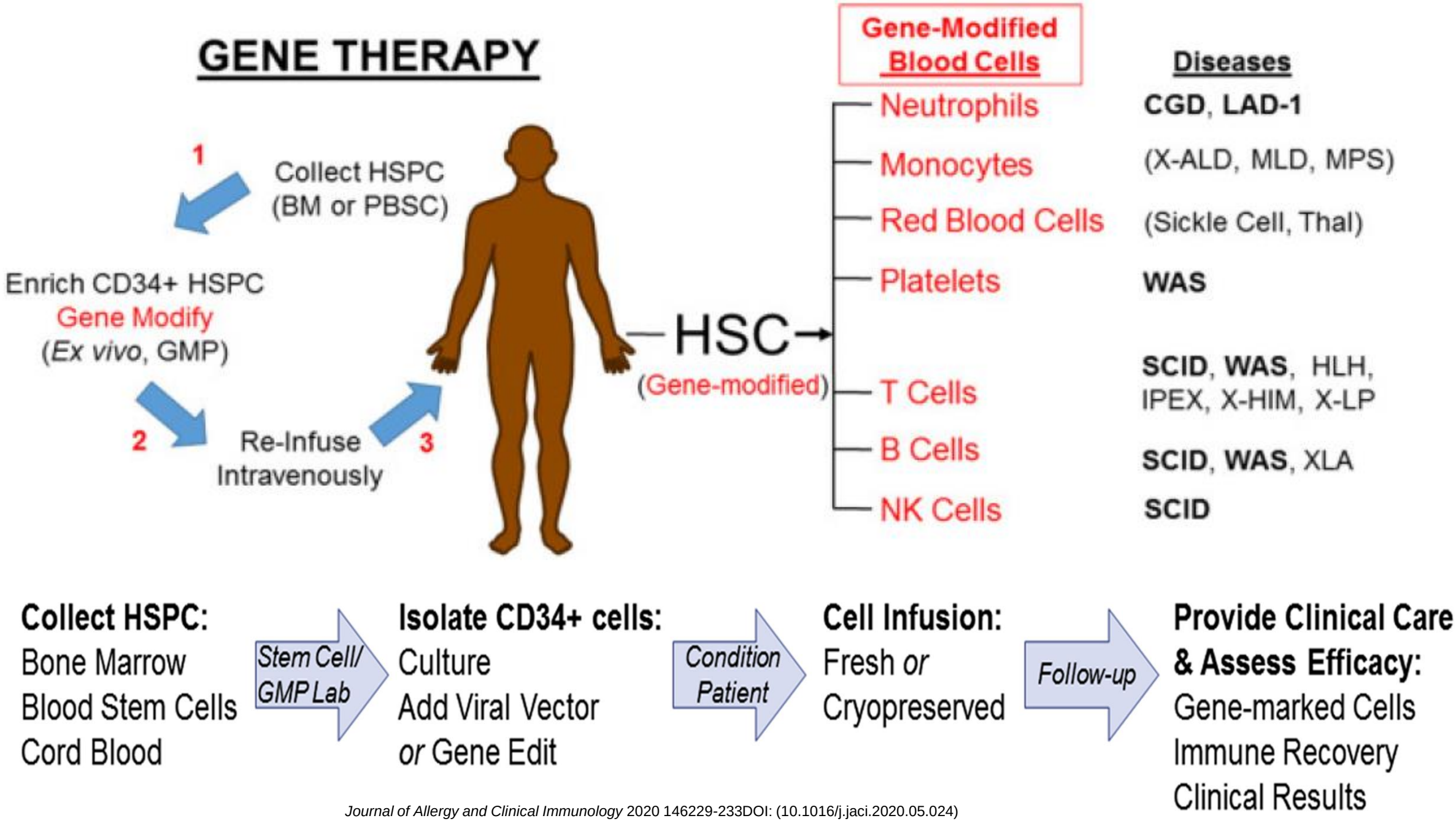
5 Return thawed genetically modified stem cells to the patient



6 Supportive care until the new stem cells engraft and immune reconstitution occurs

British Journal of Haematology, 2021, 193, 1044–1059

GENE THERAPY



Collect HSPC:
Bone Marrow
Blood Stem Cells
Cord Blood

Stem Cell/ GMP Lab

Isolate CD34+ cells:
Culture
Add Viral Vector
or Gene Edit

Condition Patient

Cell Infusion:
Fresh *or*
Cryopreserved

Follow-up

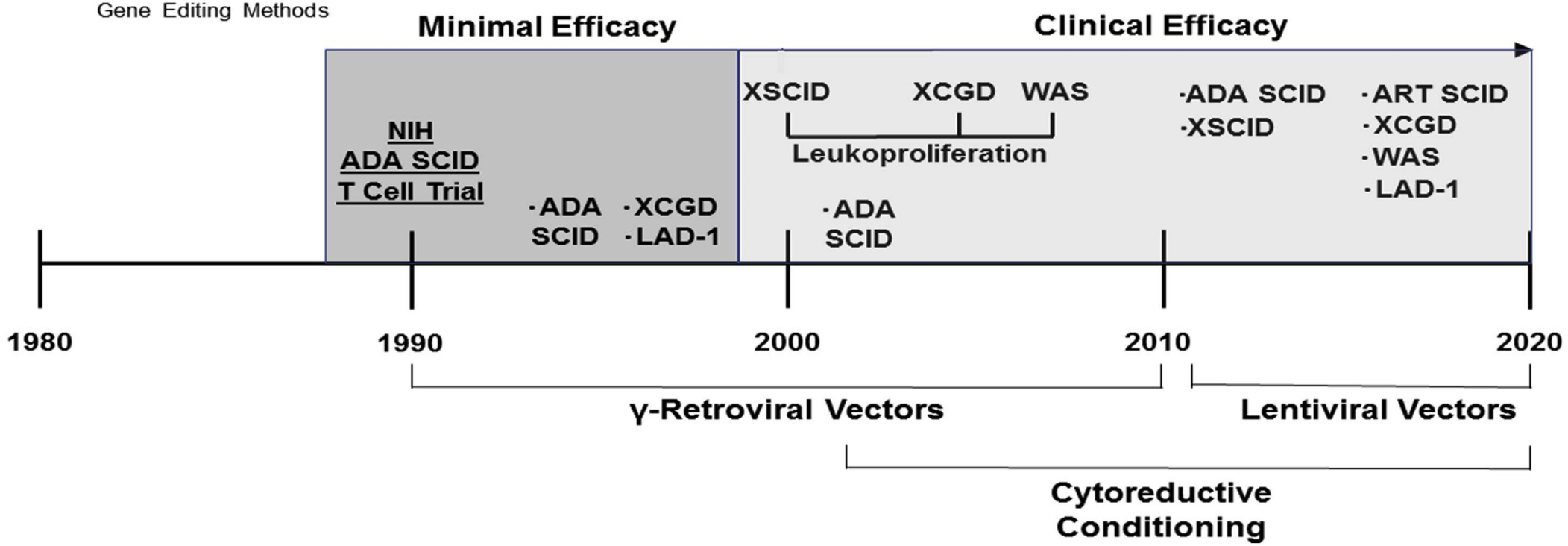
Provide Clinical Care & Assess Efficacy:
Gene-marked Cells
Immune Recovery
Clinical Results

Journal of Allergy and Clinical Immunology 2020 146229-233DOI: (10.1016/j.jaci.2020.05.024)

Timeline of Gene Therapy for Primary Immune Deficiencies

Technology Development:
Recombinant DNA Methods
Viral Vectors Developed
PID Genes Cloned
Recombinant Hematopoietic Growth Factors
CD34 Enrichment
Reduced Intensity Conditioning
Human Genome Sequence
Gene Editing Methods

The Future:
New Approaches
More Diseases Treated
More Approved Therapies



Journal of Allergy and Clinical Immunology 2020 146229-233DOI: (10.1016/j.jaci.2020.05.024)

Clinical Trials in gene therapy

- Several clinical trials have shown promising results in gene therapy for PID.
- Good results in ADA-SCID using retroviral vectors and Wiskott-Aldrich syndrome using lentiviral vectors.
- Gene therapy has improved immune function, reduced infection rates, and enhanced quality of life for patients.
- What is still unknown?
  - Optimal approach to gene correction for immune dysregulation, polyendocrinopathy, enteropathy, Xlinked, recombination activating gene 1 SCID, gain-of function mutations.
  - Relative efficacy of lentiviral vectors and gene editing approaches

TABLE I. Inherited blood cell diseases responding to hematopoietic stem cell lentiviral vector gene therapy—2020

Disorder	Clinical Trials.gov no.
PIDs	
ADA-deficient SCID	NCT01852071, NCT03765632, NCT03645460
X-linked SCID	NCT01306019, NCT03601286, NCT03311503
Artemis SCID	NCT03538899
Wiskott-Aldrich syndrome	NCT01560182, NCT01515462, NCT01347242
Chronic granulomatous disease	NCT02234934, NCT01855685, NCT02757911
Leukocyte adhesion deficiency-1	NCT03812263
Metabolic/storage disorders	
X-linked adrenoleukodystrophy	NCT01896102, NCT03727555, NCT03852498
Metachromatic leukodystrophy	NCT01560182
MPS-I (Hurler syndrome)	NCT03488394
Hemoglobinopathies	
Beta-thalassemia	NCT03207009, NCT01745120, NCT02453477
Sickle cell disease	NCT02186418, NCT03282656, NCT03964792
	NCT03964792, NCT02247843, NCT04091737

While gene therapy holds promise for improving the lives of individuals affected by genetic diseases, its availability and implementation in Africa face several challenges.

- Infrastructure: specialized laboratories, advanced medical facilities, skilled healthcare professionals…
- Cost: can be a significant barrier to access in Africa (complex option, products, manufacturing processes
- Research and Development: Lack of local research and development infrastructure hampers the ability to adapt gene therapy techniques to local needs and genetic profiles.
- Regulatory Framework: is crucial for the safe and ethical implementation of gene therapy.
- Ethical Considerations: such as informed consent, patient privacy, and equitable access, should be addressed to ensure responsible and ethical implementation

Despite these challenges, there are efforts being made to advance gene therapy in Africa. Collaborations with international institutions, training programs for local scientists and healthcare professionals, and initiatives to raise awareness about gene therapy are first steps to GT

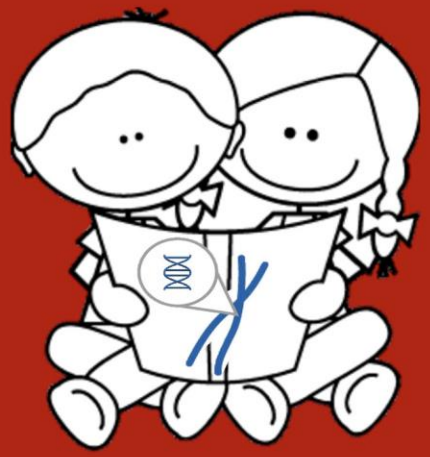
Comparison of allogeneic haematopoietic stem cell transplantation, haematopoietic stem cell gene therapy (HSC GT) using viral gene addition and HSC GT using gene editing for primary immunodeficiency.

	AlloHSCT	Autologous HSC GT (viral gene)
Donor	Requires suitably matched donor	No need for suitable donor
Conditioning	RIC regimens	Some conditioning needed
Experience	Over 50 years' experience. Good data and risks well known	Over 25 years' experience in some disease settings.
Access to treatment	Widely available in specialist transplant centers	Not widely available, can only be performed in select centres. Only one product (for ADA-SCID)
Efficacy	OS in SCID cohorts >95%. OS for other PIDs 75– 95%. Inferior outcomes in older patients (~85% in adult cohorts)	>80 patients treated, 100% survival and no genotoxicity for ADA-SCID., 10–20% re-start enzyme or had alloSCT. Lentiviral for X-SCID has minimal toxicity and reconstitutes function T and B cells. 91% survival in 34 SWA patients
Risks	GvHD Graft rejection Graft failure	Risk of insertional mutagenesis Transgene is not under the endogenous gene control machinery

Mary Slatter[1,2]* and Su Han Lum
Frontiers in Immunology,, 2023

Towards Precision Medicine and a Personalized Approach to Hematopoietic Stem Cell Transplantation and Cellular Therapy for Inborn Errors of Immunity

Early diagnosis

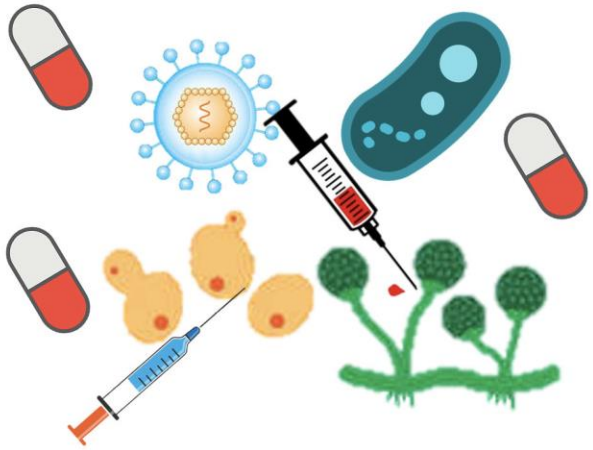


Precise molecular diagnosis

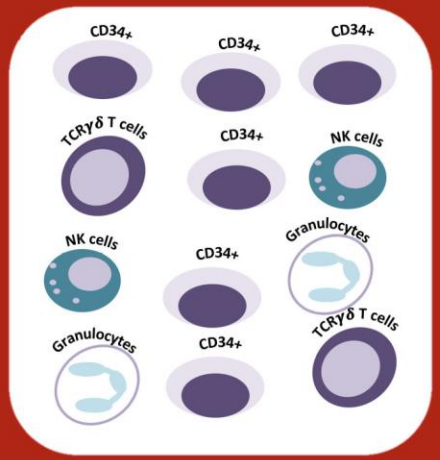
Optimizing of disease control

Targeted therapy
Monoclonal antibody

Surveillance, prophylaxis and treatment of infection prior to and during HSCT



Reduced toxicity conditioning + Individualized dosage optimization of conditioning (Pharmacokinetic study)



Precise graft prescription
Graft engineering

Post transplant care

Cellular therapy to boost immune recovery

New therapy to treat transplant-related complications

Vaccination

Survivorship program

Screening for late effects

Primary immune deficiency management challenges and hopes in Africa

Access to immunoglobulin replacement therapy and HSCT is very limited or unaffordable for many patients in some of the Africa countries

- Great discrepancies are observed in the diagnostic rate and the treatment availability for PIDs in Africa with the highest childhood DALY and mortality rates in some country
  - **Awareness about PID should be improved by:**
    - Sharing consensus clinical guidelines and experiences and exchange visits between countries
    - Collaboration including help with diagnosis, and treatment for IEI patients from countries with limited resources
    - Enhances structural clinical training, and establishes national registries and centres of excellence in PID care and HSCT in all Africa's countries
    - Increasing awareness about IEI and improved diagnostic capabilities of IEI will result in increased referrals for HSCT
    - Allocation of resources to expand the currently limited capacity for HSCT in Africa

- **ASID** was established in 2008 aiming to increase awareness and knowledge regarding PIDs in Africa by regularly conducting PID schools and congresses, and to set an African registry of PID
- **The Middle East and North Africa primary immunodeficiency network** was established to promote collaborations and experience sharing, and to produce diagnosis and Management Guidelines for IEI

ASID, The African Society of Immune Deficiency; DALY, disability adjusted life year; HSCT, haematopoietic stem cell transplant; IEI, inborn errors of immunity; PID, primary immunodeficiency.

PID diagnosis, care challenges and hopes in Africa

In countries were the two main treatment for PIDs are already available (IgRT and HSCT). Other challenges remain:

- More efforts should be made to introduce personalized therapies for patients with IEI and guarantee a sustained access to all these treatments, equally for all PIDs patients
- SubcutaneousIg,enzymatic treatment…
- Prevent early death by introducing the neonatal screening for SCID
- Enlarge PIDs networking to guarantee early diagnosis by first line physicians, prompt and adequate management of PIDs and their complications
- Facilitate access to HSCT for all PID patients and other targeted therapies to improve disease outcomes
- Create regional specialized centers in PIDs care collaborating with the National Center and expansion of the experience of HSCT to improve attainability, capacity and time between diagnosis and HSCT
- Enhance research collaborations between centers
- Gene therapy for PIDs not eligible for HSCT
- **Enhance the major role of patient organizations**

- CID, combined immunodeficiency; HSCT, haematopoietic stem cell transplant; IEI, inborn errors of immunity; Ig, immunoglobulin; IgRT, immunoglobulin replacement therapy; MEA, Middle East and Africa; PID, primary immunodeficiency; SCID, severe combined immunodeficiency.

Acknowledgements

- **Pr Mohamed Bejaoui**: the Founder of primary immune deficiency reference center in Tunisia
- All the Team of Pediatric immunopathology and hematology, and HSCT
- Departement of pharmacy BMT Center Tunis
- Laboratory, BMT Center Tunis
- National Center of Blood transfusion
- Laboratory of immunology, Institute Pasteur, Tunis
- All physiancs and Pediatricians referring PIDs in Tunisia and outside side

Patients and their families

African Society for Immunodeficiencies
Société Africaine des Déficits Immunitaires
الجمعية الإفريقية لأمراض ضعف المناعة الأولي

Pr Aziz Bousfiha

All membres of ASID
All participants in the MENA network
International collaborators
France, Germany, USA…

Departement of pediatrics: immuno-hematology and stem cell transplantation, CNGMO Tunis

Thank you for your attention

IPOPI
8TH AFRICAN PID PATIENTS' MEETING
14-15 JUNE 2023
CASABLANCA, MOROCCO
an IPOPI event

Q&R

Q&A

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PID PATIENTS'
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PAUSÉ-CAFE

COFFEE BREAK

30 min

COLLABORATION

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