



# IPOPI 4<sup>TH</sup> REGIONAL ASIAN PID MEETING

19-20 NOVEMBER 2022  
KUALA LUMPUR, MALAYSIA

an **IPOPI** event

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# The value of registries in PID

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# Why do we need registries?

## **Epidemiology**

- Prevalence
- Incidence

## **Diagnostic rate**

Under/Over-diagnosis (All PID or Specific PIDs i.e. CVID, SCID...)

Calendar trend in diagnostic delay (All PID or Specific PIDs i.e. CVID, SCID...)

# Why do we need registries?

## **Natural history of PIDs**

### **Comorbidities:**

Infections

Autoimmunity

Inflammation

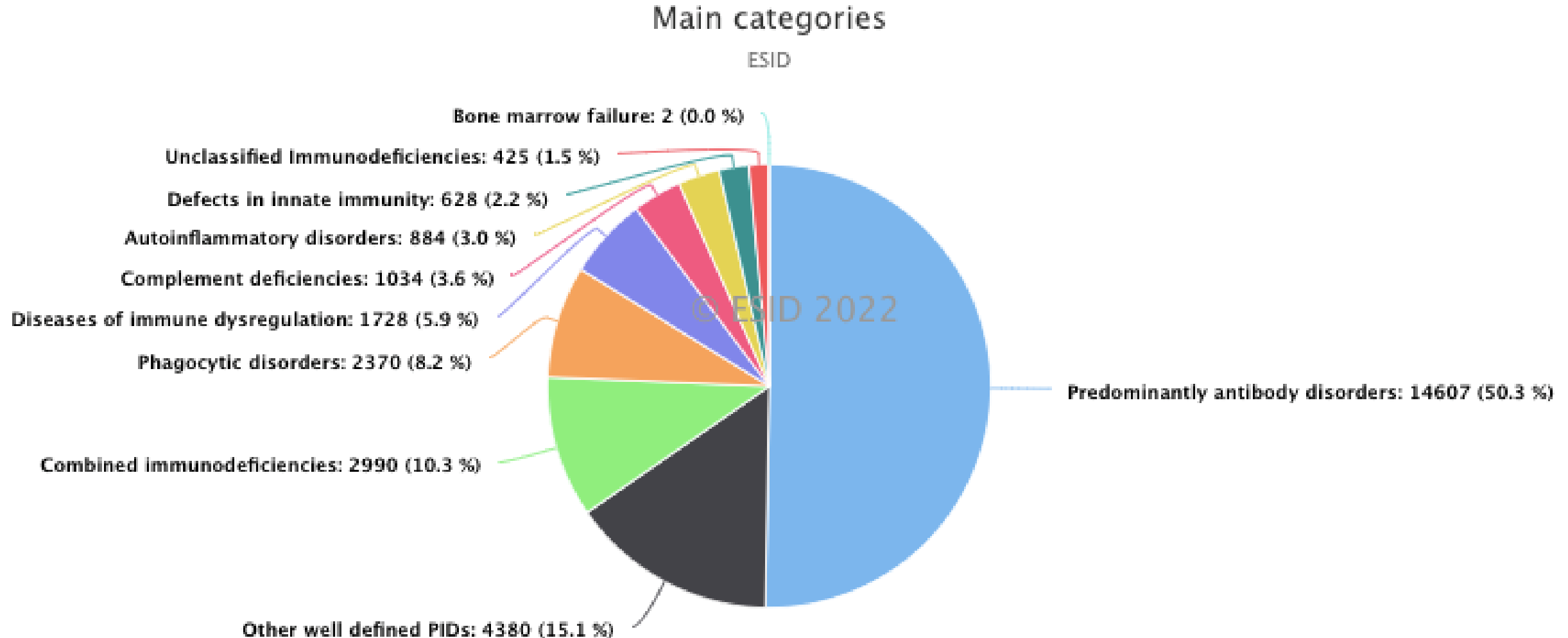
Malignancies

Other

Prognosis

## **Description of main therapies (IgRT, HSCT, other...)**

# ESID Registry : 29342 patient registries



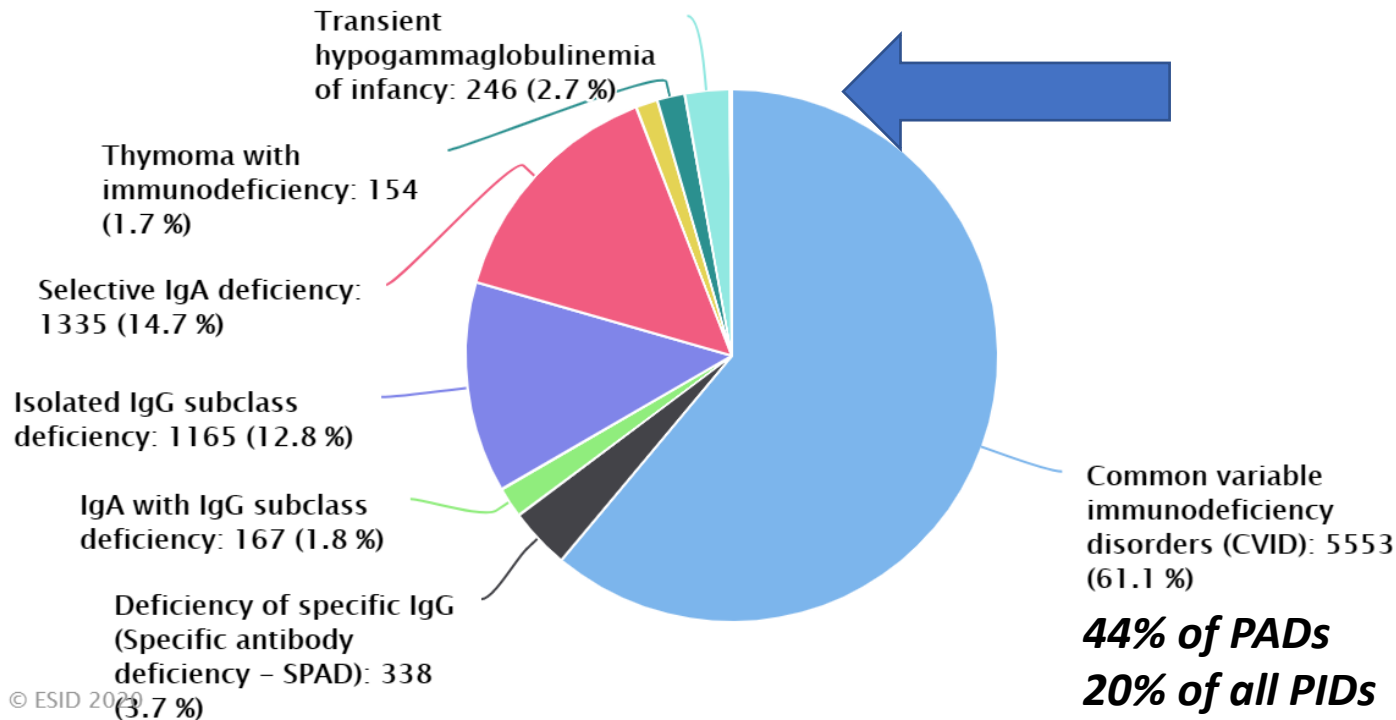
# Specific diagnosis

PID main categ

ESID

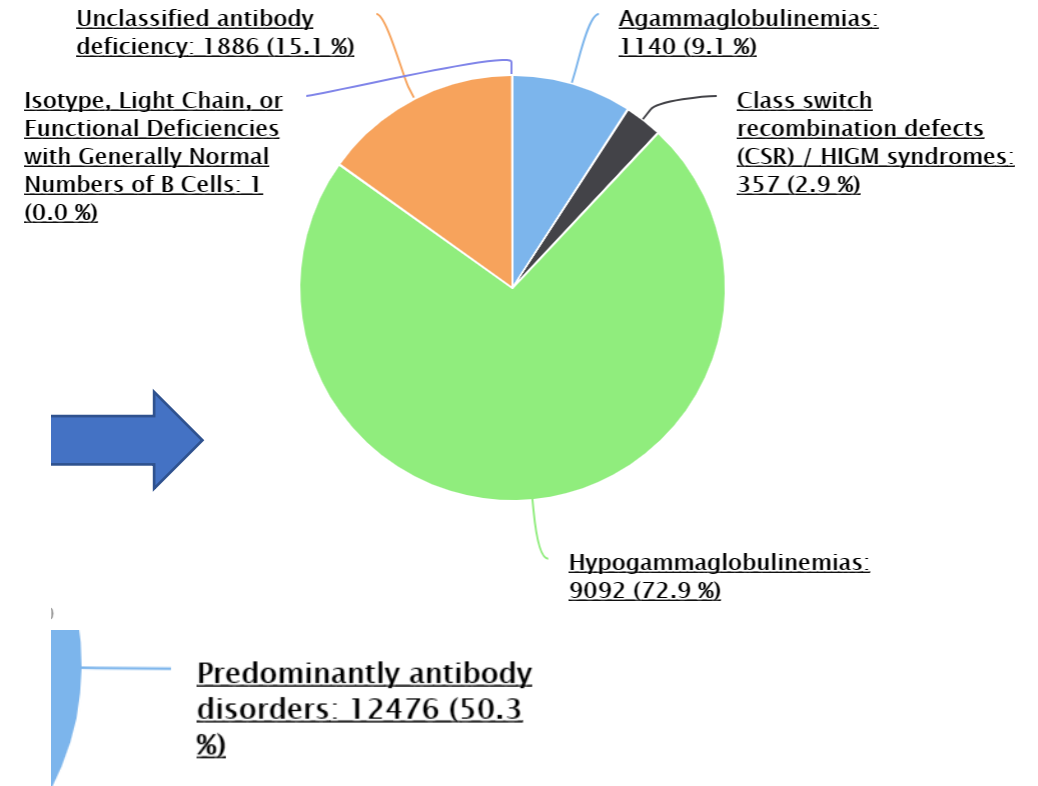
## PID diagnoses of Hypogammaglobulinemias

ESID



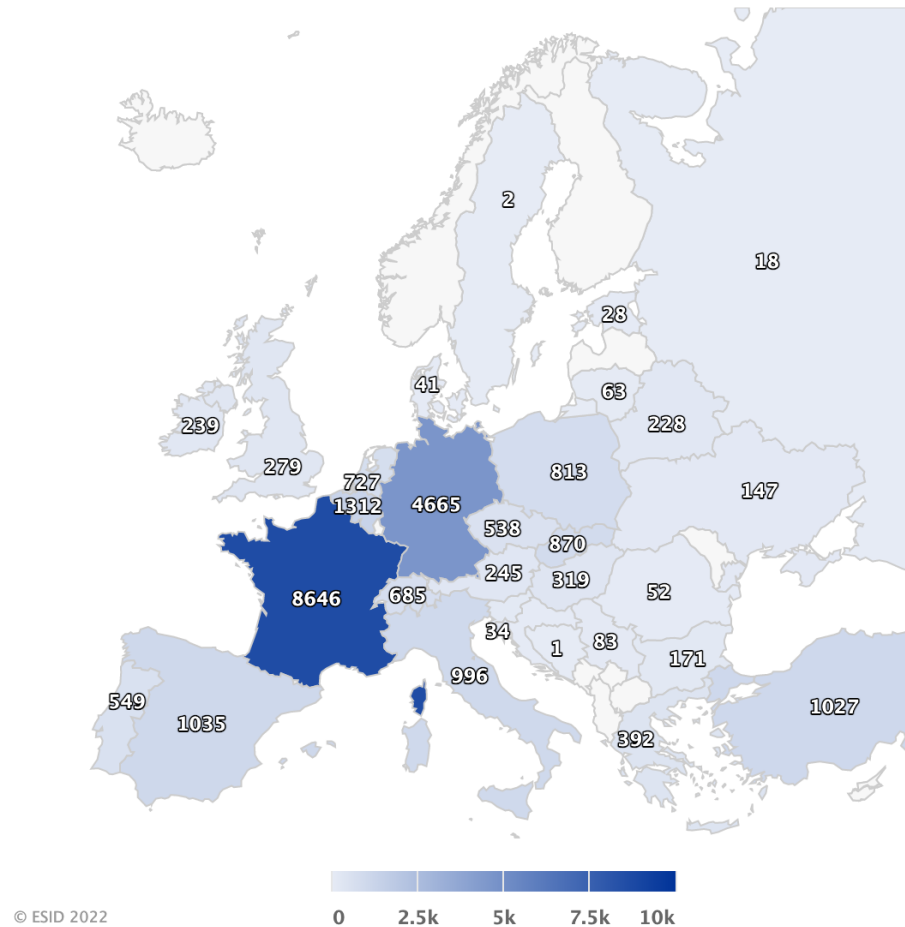
## Subcategories of Predominantly antibody disorders

ESID



# Registered patients per country

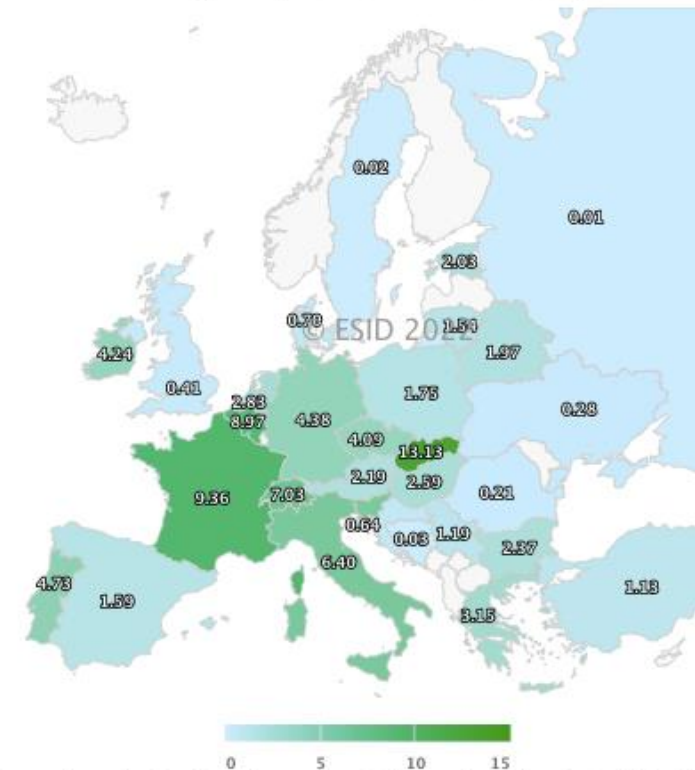
Registered patients per country  
including patients documented as deceased



Europe Only participating

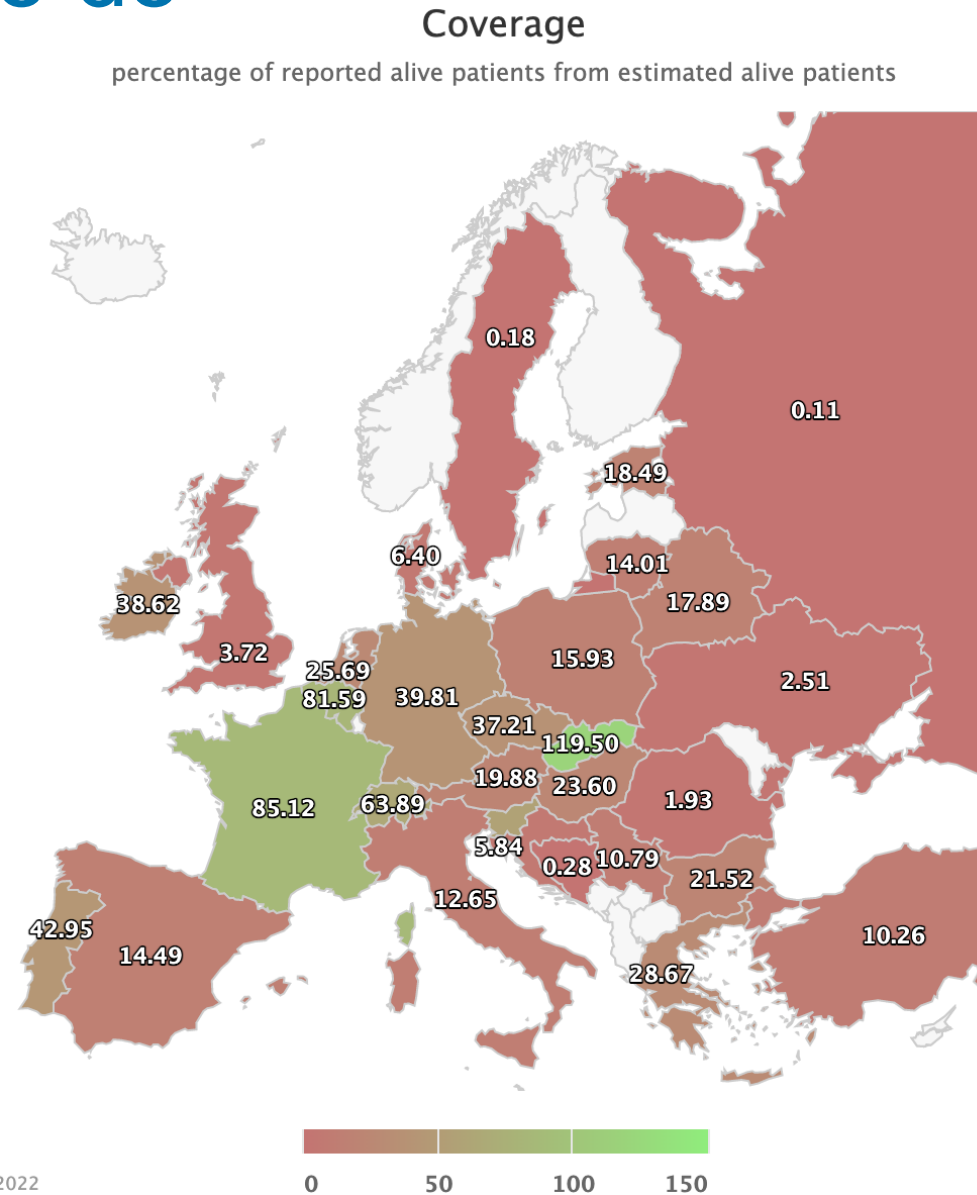
Prevalence Map

Prevalence Map  
Living patients per 100000 inhabitants (N = 23543)

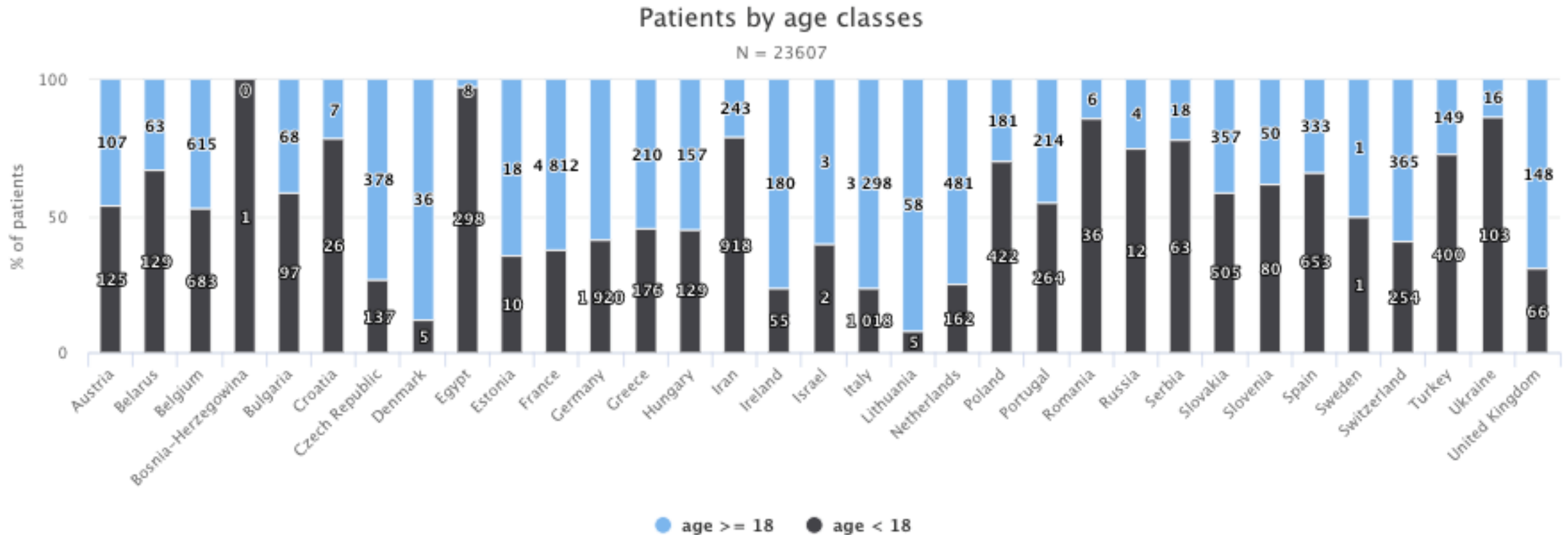


The map shows the minimal prevalence rates calculated from the number of documented PID patients in the ESID Database.

# Some work to do



# Age distribution

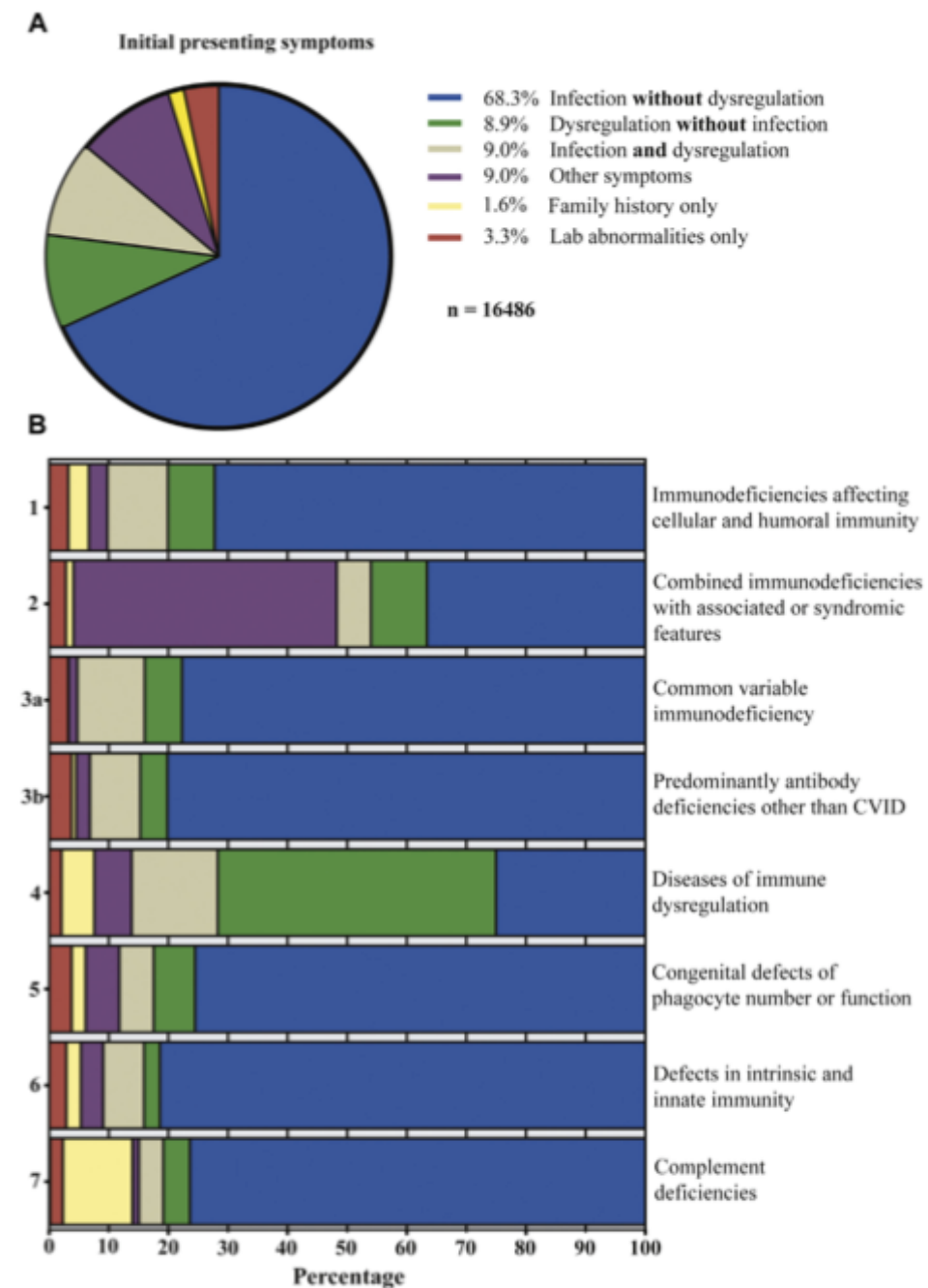
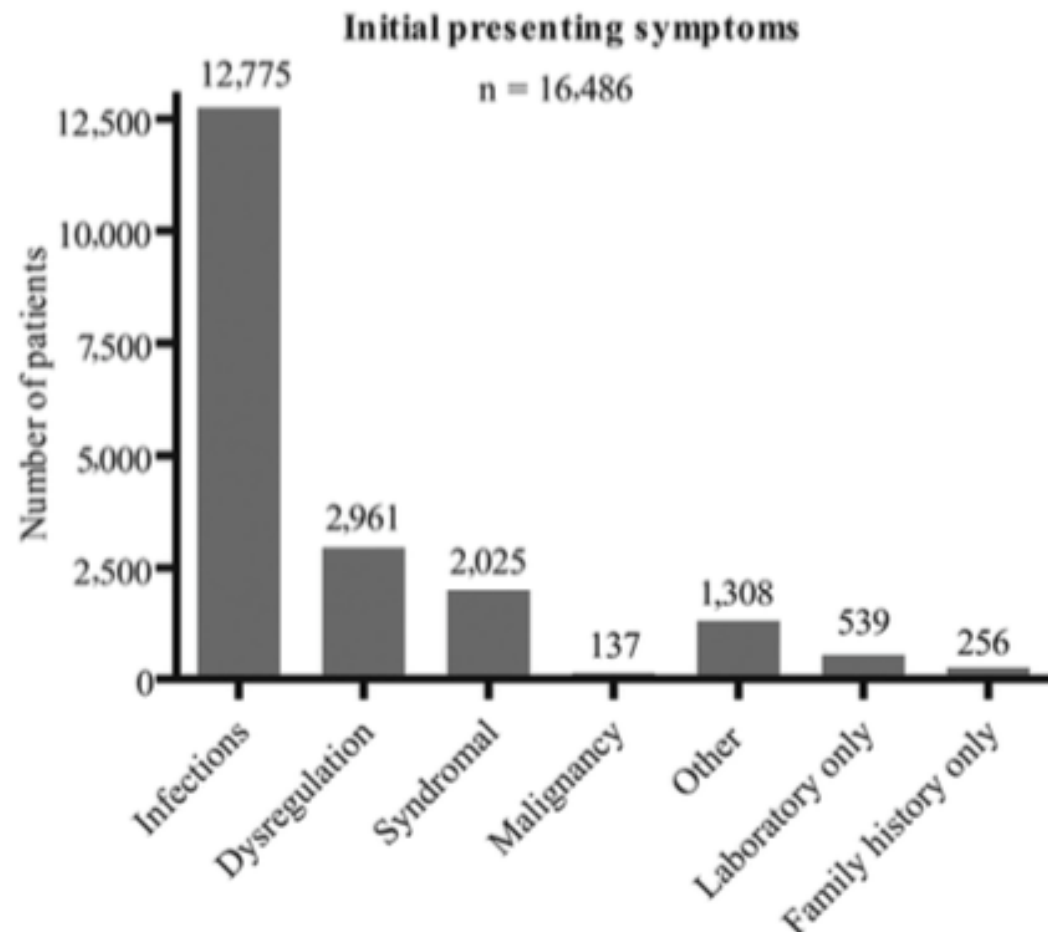


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We calculate the age of a patient by computing the distance between date of last news minus date of birth.

# Initial presenting manifestations in 16,486 patients with inborn errors of immunity include infections and noninfectious manifestations

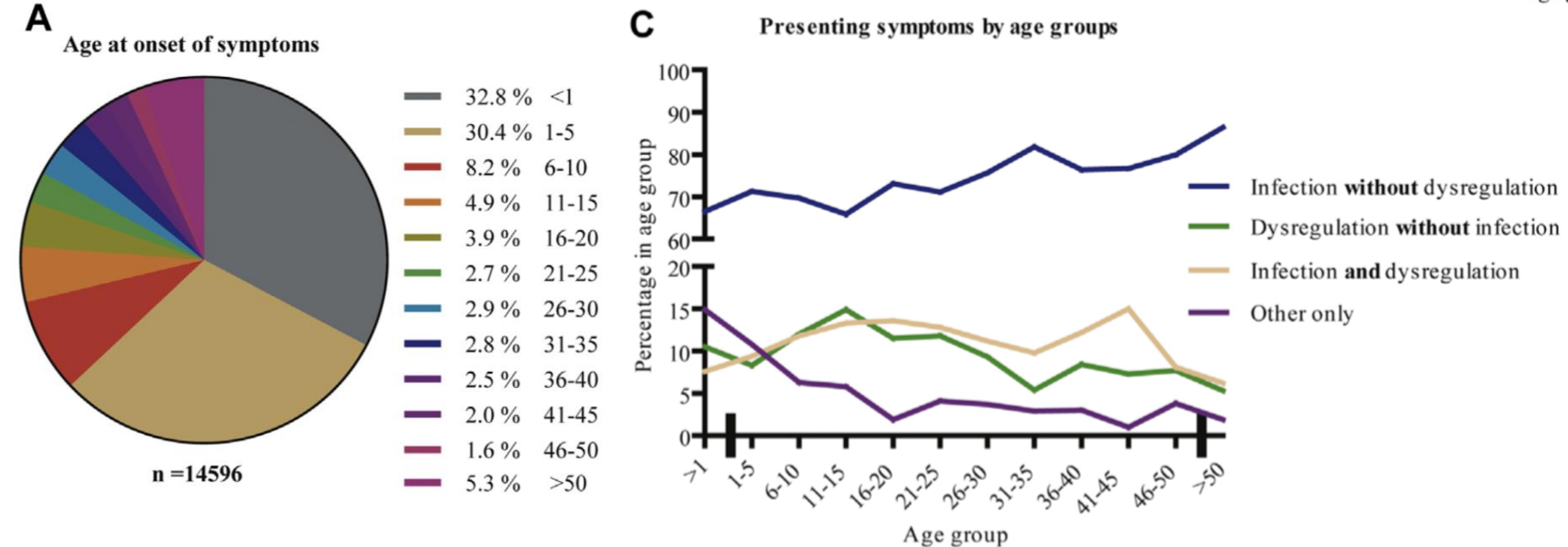
Julian Thalhammer, MD,<sup>a,b,\*</sup> Gerhard Kindle, MD,<sup>a,\*</sup> Alexandra Nieters, PhD,<sup>a</sup> Stephan Rusch,<sup>a</sup> Mikko R. J. Seppänen, MD,<sup>c</sup> Alain Fischer, MD,<sup>d,e</sup> Bodo Grimbacher, MD,<sup>a,f,g,h</sup> David Edgar, FRCP, FRCPATH,<sup>i,j</sup> Matthew Buckland, MD,<sup>k,l</sup> Nizar Mahlaoui, MD, MPH, PhD,<sup>d,e</sup> and Stephan Ehl, MD,<sup>a,g</sup> for the European Society for Immunodeficiencies Registry Working Party<sup>‡</sup> *Freiburg, Germany; Helsinki, Finland; Paris, France; Dublin, Ireland; and London, United Kingdom* <https://doi.org/10.1016/j.jaci.2021.04.015>



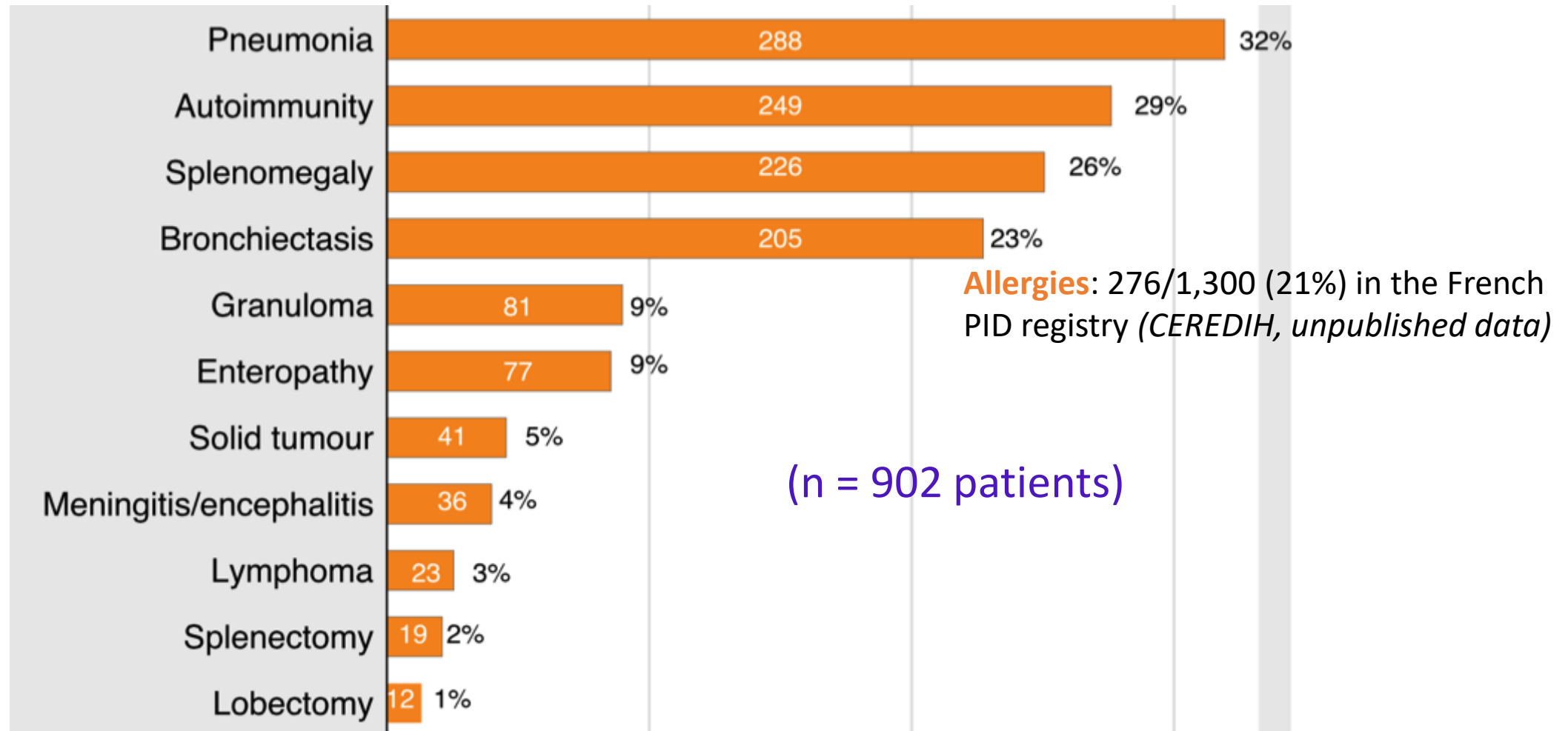
**FIG 3.** Pattern of initial presenting manifestations. **A**, Patterns of initial presenting manifestations in the complete cohort. "Other" summarizes initial presentation with syndromic features, malignancy, or other symptoms. **B**, Patterns of initial presenting manifestations by IEL disease groups.

# Initial presenting manifestations in 16,486 patients with inborn errors of immunity include infections and noninfectious manifestations

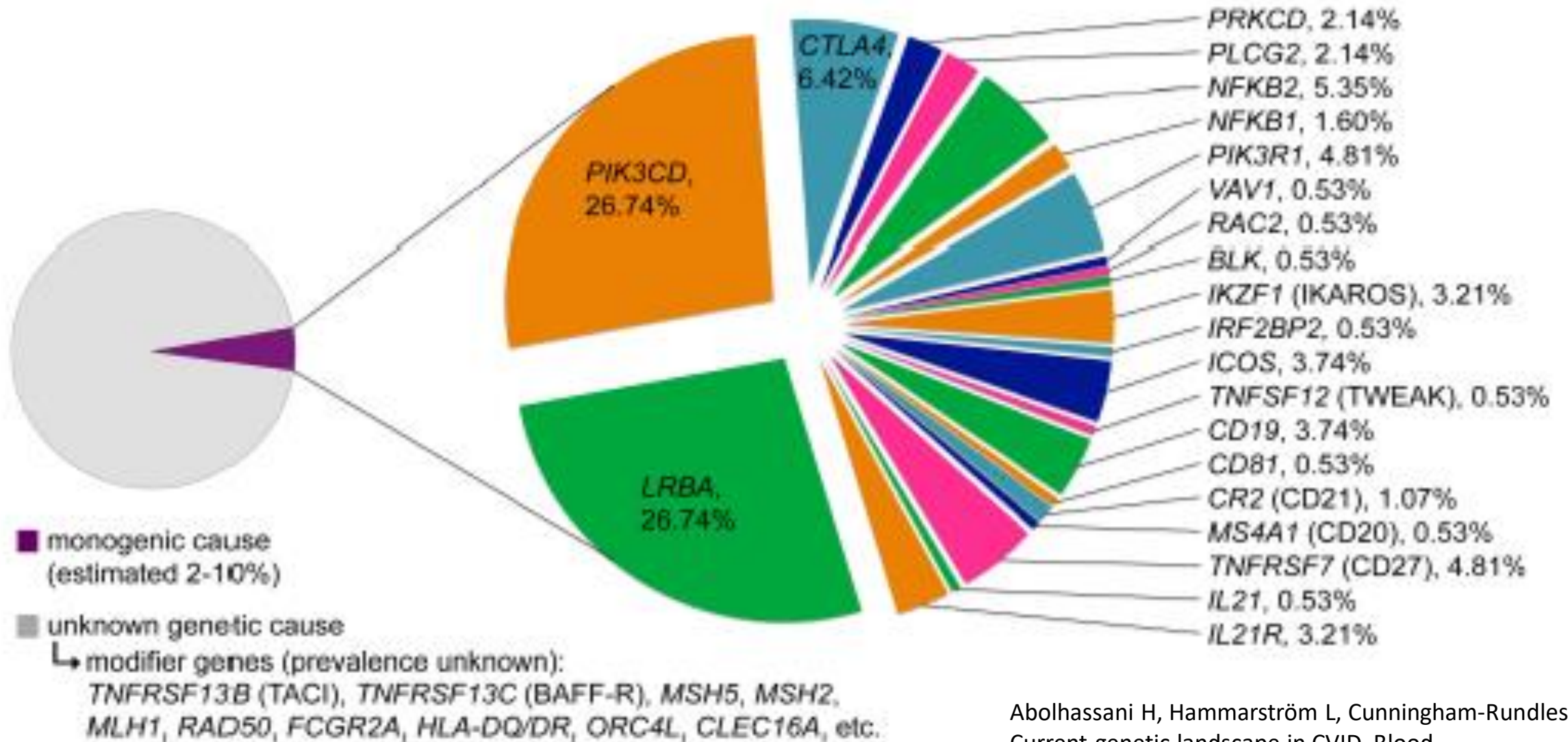
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# Frequency of clinical features in PID



# Genetic defects in PID



Abolhassani H, Hammarström L, Cunningham-Rundles C.  
 Current genetic landscape in CVID. *Blood*.  
 2020 Feb 27;135(9):656-667.

# Insights in treatments used



# Goals

- Health economics
  - Allocation/Planification of resources
    - Diagnosis: laboratories (immunology, microbiology, pathology...) + Genetics
    - Therapies: IgRT, HSCT, Targeted therapies...
  - Need to train medical students/young doctors
  - Need for
    - Hospital beds (Ig infusions)
    - Adult medicine units (transition of care from pediatrics to adult care)
    - HSCT units
    - Newborn screening
- Promote
  - Education
  - Awareness

# Challenges

- Informed consent
- Unified eCRF
- Governance
  - Steering Committee
  - Regulated access to the database for data entry and for study proposals
  - Publication rules
- IT team
- Data Management and Quality controls
- Trained staff in healthcare units
- Reporting (newsletter, website)
- Sustainability

# Thank you



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