2ND meeting of the European Research Consortium on ITP

NEW INSIGHTS INTO IMMUNE THROMBOCYTOPENIA

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Artificial intelligence & machine learning for diagnosis & management of ITP

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Disclosures

Company name	Research support	Employee	Consultant	Stockholder	Speakers bureau	Advisory board	Other
Novartis	X				X		
Amgen	X				X		
MedImmune			X				
SOBI			X		X	X	
Agenx			X			X	
Takeda			X			X	
Dexel Pharma			X				
ClimbBio			X				

Where are we with ITP?

It is biologically complex … and increasingly data-rich

It is now as much a **data problem** as a biological one

Using traditional clinical approaches we are not going to move forward—limit reached

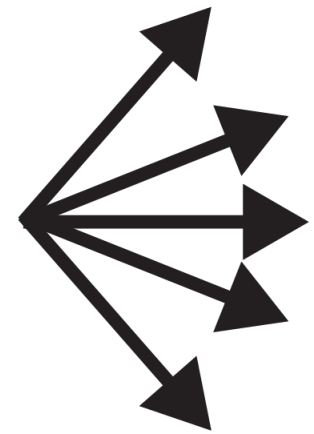
AI is not optional—it is required

?

ITP

FOCUS

New therapies



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Current practice … educated guess

ITP should be solvable

- understand much of the biology
- we have effective treatments
- outcomes often good

But we cannot answer basic questions

Most treatments developed in the dark

Questions we still can’t answer reliably

01

How do we know it’s ITP?

No definitive diagnostic test — diagnosis remains one of exclusion

02

Patient “risk” level?

Risk stratification beyond platelet count is imprecise and subjective

03

Which treatment is best for this patient? For how long?

No validated biomarkers guide personalised therapy selection

These three challenges remain difficult — or impossible — with current technologies alone

Major unmet needs

1 Definitive diagnosis

2 Risk stratification (avoidance of over-treatment)

3 Treatment choice & personalisation

Difficult (impossible) with current technologies

AI and machine learning may be the answer …

Artificial intelligence/machine learning are key tools

Analyse large amounts of data quickly

Major advances in other disease areas

May help with some of our current issues in ITP

AI & ML already making impact

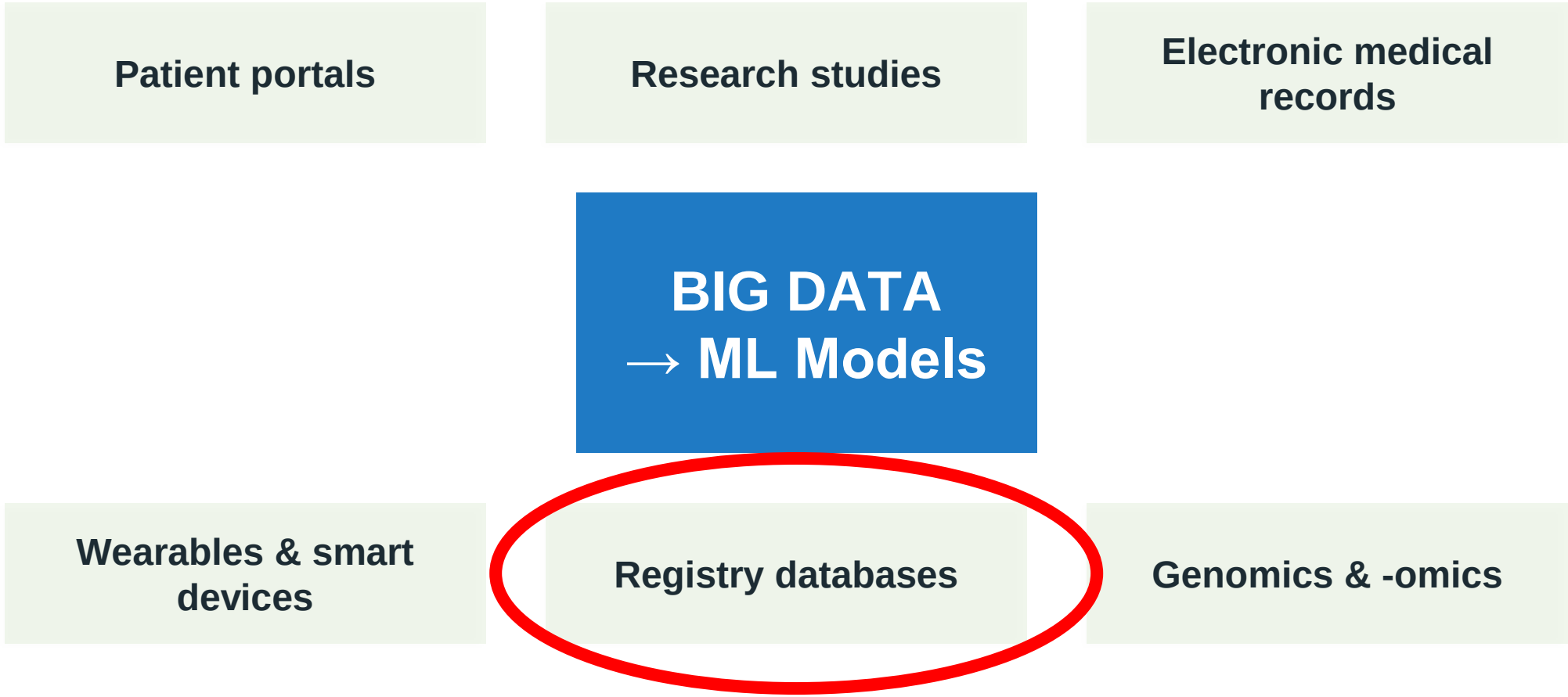
DIAGNOSTICS

- Radiology & imaging
- Digital pathology
- Endoscopy & ultrasound
- Biochemistry & haematology
- Blood film analysis

MANAGEMENT

- Disease prediction & prognosis
- Personalised treatment selection
- Patient monitoring
- Genetic & multi-omic analysis
- Automating routine clinical tasks

What AI needs: big data



Registries are the most valuable structured data source in ITP

Registries are most important sources of data

ITP registries: 25+ years of longitudinal data

UK ITP Registry

Adult primary, secondary, paediatric, pregnancy cohorts
(Barts/QMUL)

Carmen Registry

European multicentre; treatment outcomes & QoL
(European collaborative network)

ICIS Registry

International ITP — global multicentre dataset
(International ITP Study Group)

Other national registries

US, Nordic, Asia-Pacific, others

25+ years longitudinal follow-up.

>10,000 patients registered globally

Pifalls with registries

What we choose to measure/record

Are they standardised? Unlikely

Data quality

Never designed to uncover complex biological problems

AI in haematology

How well are we doing?

AI in haematology: the broader context

Haemato-oncology is well ahead — ITP lagging behind

Haemato-oncology

AML/MDS — mutation classificn & prognosis

CML — response prediction

CLL — AI models for treatment guidance

Myeloproliferative neoplasms

Non-malignant haematology

Thalassaemia — diagnosis & genotyping

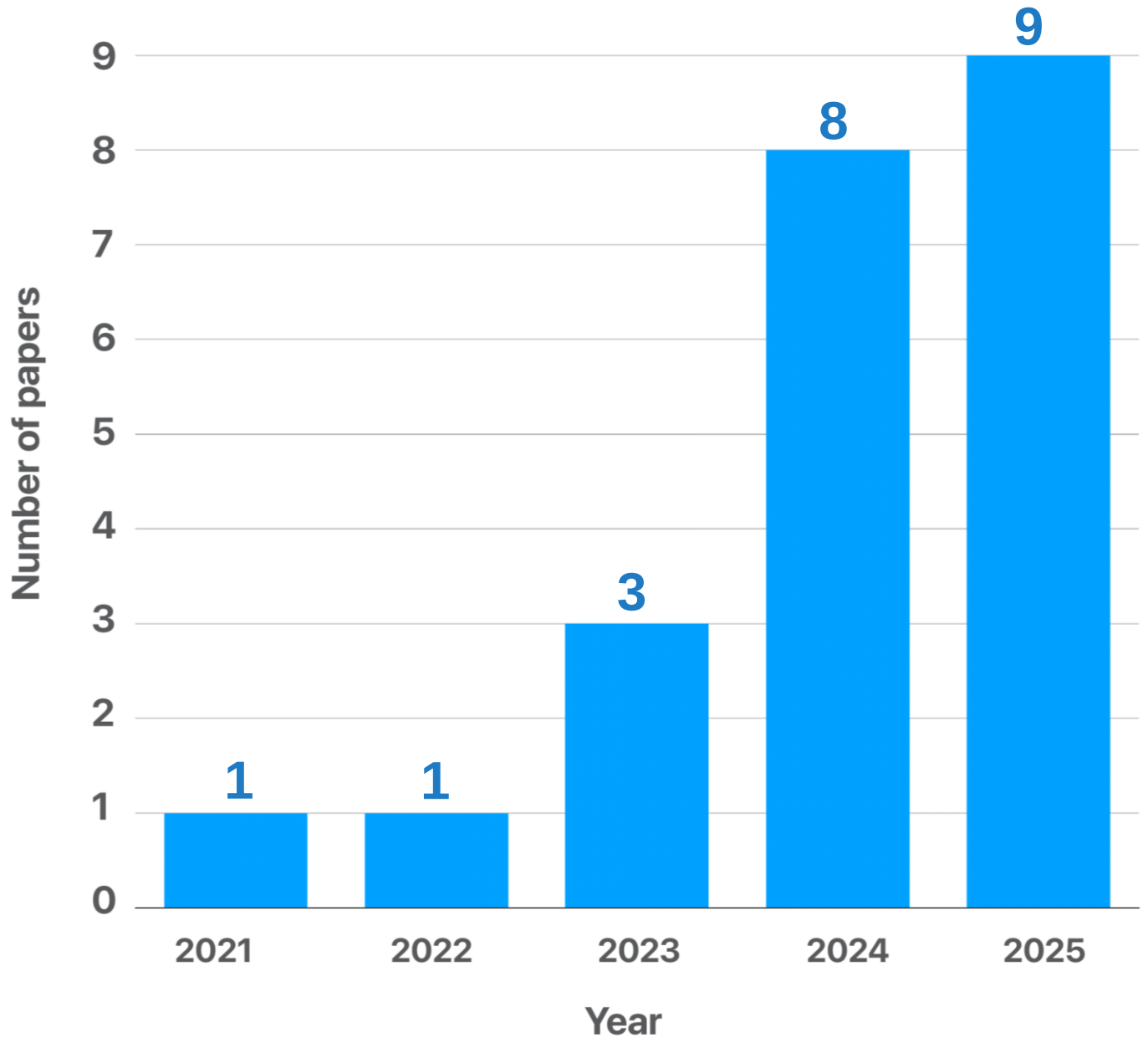
Sickle cell disease — complication prediction

Iron deficiency anaemia — ML prediction

Thrombocytopenia — general classification

AI & machine learning in Primary ITP

Papers per year



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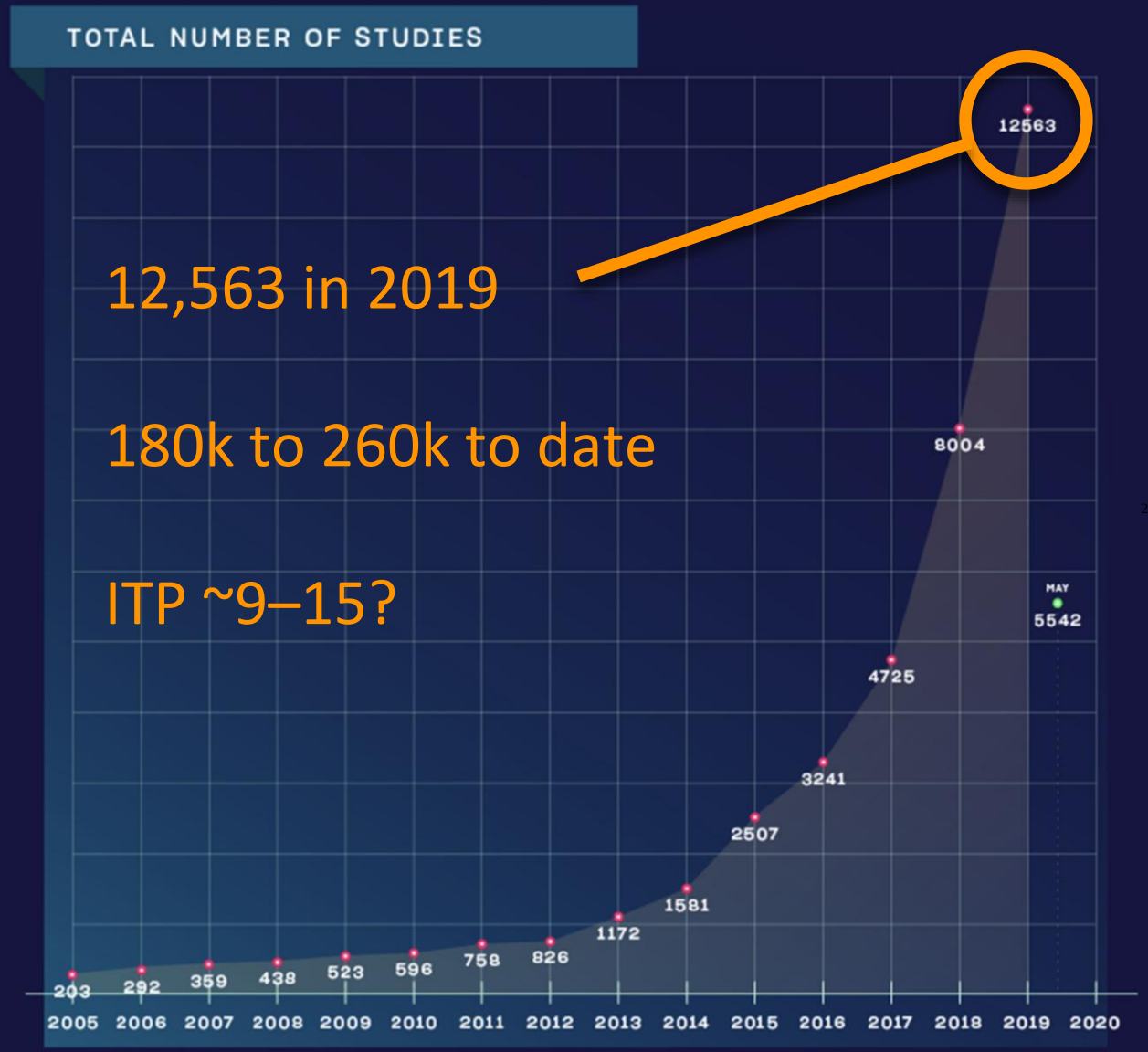
MACHINE AND DEEP LEARNING STUDIES ON PUBMED.COM



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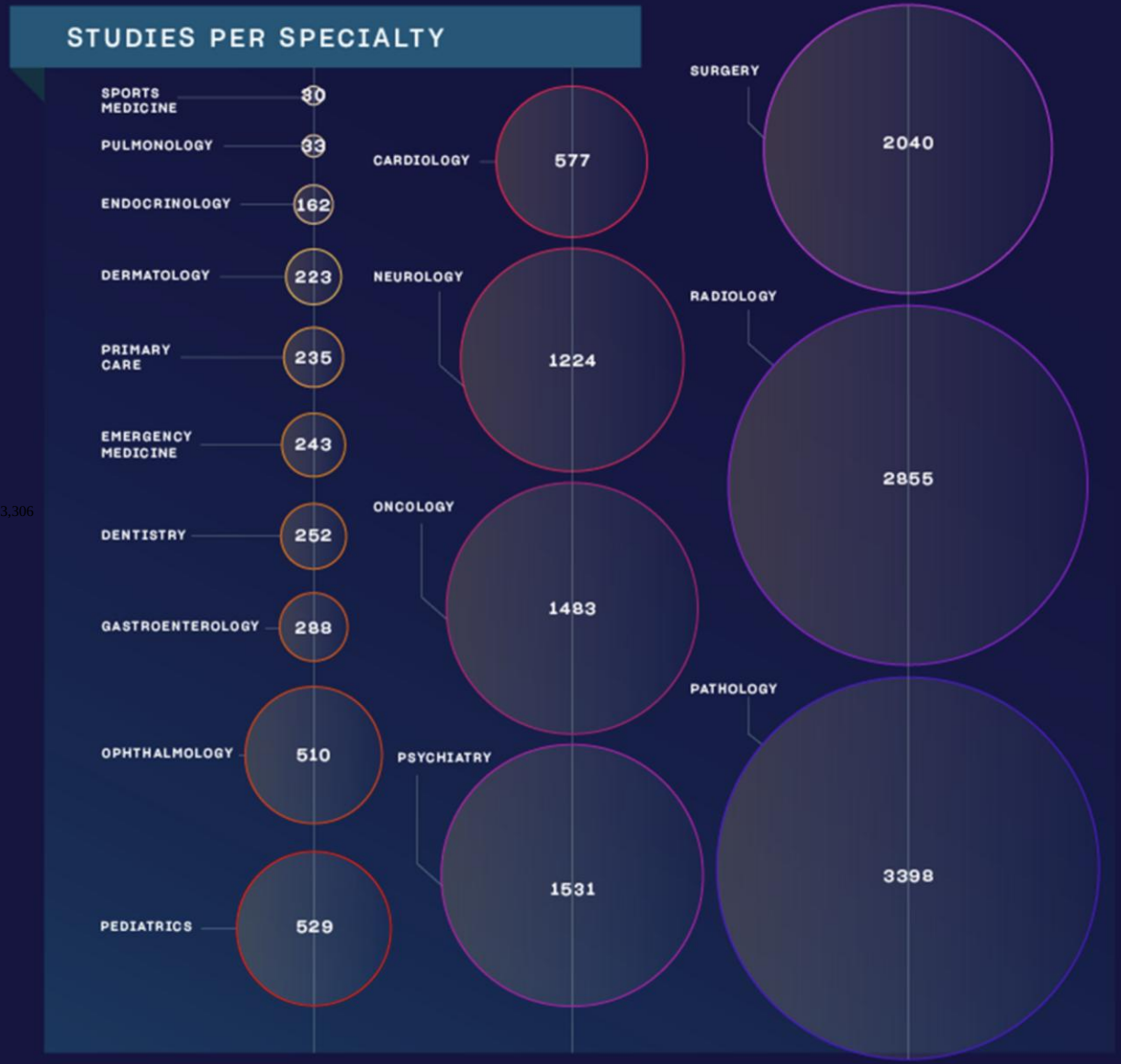

AI in ITP: Key Studies

Diagnosis · Bleeding risk · Treatment prediction

Key studies to date (2023–2025)

Study	Focus	Population	Best model	AUC
1. Wen et al 2024	Diagnosis + bleeding	77 + 62 pts	RF + SVM-RFE	0.82–1.00
2. Miah et al 2024	Diagnosis feasibility	Registry data	ML classifier	—
3. An et al 2023	Bleeding risk (adult)	Nationwide cohort	Personalised ML	0.85
4. Shen et al 2025	Bleeding risk (paed)	286 children	XGBoost	0.89
5. Ma et al 2024	Chronicity (paed)	662 children	XGBoost	0.85
6. Ma et al 2025	Ritux response (paeds)	156 children	MLP	0.88
6. Xu et al 2024	Treatment response	506 adults	XGBoost/ANN/DT	0.89–0.96

Diagnosis

Adult ITP

DIAGNOSIS

1. Wen et al: chemokines + ML for ITP diagnosis

STUDY DESIGN

40-plex Luminex chemokine profiling

Discovery (n=77) + Validation (n=62) cohorts

ML: Random Forest + SVM-RFE feature selection

ROC analysis — diagnosis & bleeding risk

KEY RESULTS

Diagnosis model

5 chemokines: ↑CCL20; ↓IL-2, CCL26, CCL25, CXCL1

AUC = 0.82–1.00 (excellent discrimination)

Bleeding risk model

6 chemokines: ↑CXCL8, CCL21, CXCL10, CCL8, CCL3, CCL15

AUC ≈ 0.72–0.79 — bleeders vs non-bleeders

Replicated in independent validation cohort

Wen Q et al. Br J Haematol. 2024;205:1938–1950

1. Wen et al: chemokines + ML for ITP diagnosis

Conclusion

Distinct **chemokine** signatures, refined by machine learning, can improve **diagnosis** of primary ITP and **predict bleeding** risk

These biomarker panels could complement platelet counts and clinical scores to guide management, pending multicentre validation

Wen Q et al Brit J Haem 205, 1938-1950 (2024)

DIAGNOSIS

2. Miah et al — can ML assist ITP diagnosis?

A feasibility study using registry-derived clinical data

Aim

Test whether ML can distinguish primary ITP from other thrombocytopenias using routine haematological variables

Approach

Applied ML classifiers to registry data; assessed sensitivity/specificity for ITP vs non-ITP

Significance

First UK-based ML feasibility study in ITP — directly relevant to NHS practice and Barts registry data

Next steps

Larger prospective cohort needed; integration of immunological and molecular data planned

Miah H, Kollias D, Pedone GL, Provan D, Chen F. Diagnostics. 2024;14:1352

Predicting Bleeding Risk

Adult & paediatric ITP

3. An Z-Y et al

A life-threatening bleeding prediction model for immune thrombocytopenia based on personalized machine learning

An Z-Y et al Sci Bull 68, 2106-2114 (2023)

3. An et al — life-threatening bleeding in adult ITP

Nationwide prospective cohort · Personalised ML model · Sci Bull 2023

AIMS

Predict life-threatening bleeding events at individual patient level; move beyond platelet count alone

APPROACH

Personalised ML framework applied to nationwide Chinese ITP registry; multiple candidate predictors including platelet dynamics, treatment history, demographics

KEY FINDINGS

AUC 0.85 (strong predictive accuracy) for life-threatening bleed prediction
Personalised model outperformed traditional scoring

Suggests early intensification for identified high-risk patients

An Z et al. Sci Bull (Beijing). 2023;S2095-9273(23)00516

4. Shen X et al

Prediction of moderate-to-severe bleeding risk in pediatric immune thrombocytopenia using machine learning

Shen X et al Eur J Pediat 184, 283 (2025)

4. Shen et al — moderate-to-severe bleeding in paediatric ITP

STUDY DESIGN

- 286 children (<18 y) — Chongqing, 2022–24
- 13 predictors → 8 by LASSO selection
- Predictors: age, platelet count, lymphocytes, preceding infection, bleeding grade
- 7 algorithms compared (XGBoost, RF, SVM…)
- 80:20 split + 10-fold cross-validation
- SHAP for interpretability

RESULTS

0.886
AUC (XGBoost)

86%
Accuracy

Top predictors:

- Older child age
- Younger age at diagnosis
- Low initial platelet count
- Excellent calibration — decision-curve analysis confirmed clinical utility

Shen X et al. Eur J Pediatr. 2025;184:283

Chronicity Prediction

Paediatric chronicity · Adult treatment personalisation

5. Ma J et al

Machine-learning models developed and internally validated for predicting chronicity in paediatric immune thrombocytopenia

Ma J et al JTH 22, 1167-1178 (2024)

CHRONICITY

5. Ma et al — predicting chronic ITP in children

662 children · Beijing Children's Hospital 2018–21

METHODS

- 16 predictors: T-cell subsets + age + sex
- Th1, Th2, Th17, Treg, Th17/Treg ratio, TCRγδ⁺ T, double-negative T
- 4 algorithms: LR, RF, SVM, XGBoost
- 70:30 split + 5-fold cross-validation

RESULTS

0.85

XGBoost AUC (best)

80%

Accuracy

Chronic ITP risk profiles:

- High risk: age >11y + Th17 ≥0.55% + TCRγδ⁺ ≥20.9% → 85% chronicity
- Low risk: age <6y + Th17 <9.6% → <20% chronicity

Ma J et al. J Thromb Haemost. 2024;22:1167–1178

5. Ma et al — predicting chronic ITP in children

Conclusion

An XGBoost model using routine immunologic markers **accurately predicted chronicity** at diagnosis in children with primary ITP

Age and Th17/Treg imbalance were dominant risk factors

External, multicentre validation is required before clinical application

Ma J et al JTH 22, 1167-1178 (2024)

Treatment response prediction

TREATMENT PREDICTION

6. Ma et al — predicting rituximab response in children

156 children · Beijing Children's Hospital 2020–23 · Br J Haematol 2025

METHODS

- 73 variables → 25 by recursive feature elimination
- 4 models: LR, RF, XGBoost, MLP (neural network)
- 5-fold cross-validation; SHAP for interpretability
- Top predictors: ANA titre, thyroglobulin Ab, steroid response, bleeding severity (positive); disease duration, IL-17^{+} T cells (negative)

RESULTS

MLP (best)	**AUC 0.88**
Logistic reg	AUC 0.78
XGBoost	AUC 0.77
Random Forest	AUC 0.73

Ma J et al. Br J Haematol. 2025;207:463–474 (***SHAP**: Shapely Additive exPlanation; **MLP**, multilayer preceptron)*

TREATMENT PREDICTION

Xu et al — personalised scoring for adult ITP treatment

506 adults + 108 external validation · Wuhan 2018–23 · Br J Haematol 2024

Glucocorticoids

Best model: XGBoost

AUC 0.89

Score threshold:
≥3 = poor response

Predictors: PLT, IP-10, TNF-α, Treg%, B cells

TPO-RAs

Best model: Decision Tree

AUC 0.80

Score threshold:
≥5 = poor response

Predictors: PLT, TGF-β1, MCP-1, IL-21, Th1%, Treg%, TPO, MK count

Rituximab

Best model: Neural Network

AUC 0.79

Score threshold:
= 3 = poor response

Predictors: IL-12, Breg%, MAIPA status

Xu M et al. Br J Haematol. 2024;205:1108–1120

TREATMENT PREDICTION

Xu et al — personalised scoring for adult ITP treatment

Score each factor (1 point each) → total score predicts likelihood of treatment failure

GLUCOCORTICOIDS

Score ≥3 = poor

- PLT ≤10 ×10^9/L
- Treg <4%
- B cells ≥22.2%
- IP-10 ≥138.9 pg/ml
- TNF-α ≥212 pg/ml

TPO-RAs

Score ≥5 = poor

- Th1 ≥25%
- TGF-β1 ≤253 pg/ml
- MCP-1 ≥167 pg/ml
- IL-21 ≥111.4 pg/ml
- MK ≥139/microslide
- TPO ≥72.3 pg/ml
- +2 others

RITUXIMAB

Score = 3 = poor

- Breg <3.8%
- IL-12 ≥195 pg/ml
- MAIPA negative

Xu M et al. Br J Haematol. 2024;205:1108–1120. ***IP-10****, Interferon gamma-induced protein 10 = CXCL10*

Promising … but still need

- Ethical standards
- Standardisation
- Improved knowledge for health care professionals
- Get patients used to AI

and much more …

AI in ITP: conclusions

These early studies show value in diagnosis, bleeding risk, and treatment prediction

Models outperform traditional scores in small cohorts — **proof of concept achieved**

Challenges & Next Steps

What needs to happen before AI enters the clinic

Diagnosis

Cannot have a single diagnostic test for a disease that is not a single disease

AI will not give one diagnostic test

Will provide classification of ITP into **subtypes**

Subtype:

- own diagnostic signature
- own treatment pathway

Replace trial and error with **subtype → diagnosis → targeted treatment**

CHALLENGES

Current limitations — common to all published models

Single-centre data

Most studies from one or two Chinese centres — ethnic, clinical and healthcare-system variation limits generalisability

Small cohorts

N = 77–662: insufficient for robust external validation or discovery of rare subgroups

Internal validation only

Most models validated on a held-out subset of the same dataset — susceptible to overfitting

Immunological features not routine

Chemokine panels, T-cell subsets, MAIPA — require specialised assays not available in most centres

Ethical & equity concerns

AI trained on limited demographics may perform poorly in under-represented populations

Clinician literacy

Most haematologists have limited ML training — black-box outputs reduce trust and uptake

We need

Large standardised registries

Linked biobanks

Immune profiling

Genomic data

Treatment outcomes

Patient-reported outcomes

No single centre has this — needs collaborative approach

AI in ITP: Conclusions

- AI is moving fast — ITP is lagging behind the broader haematology field
- Proof of concept achieved: early models show value in diagnosis, bleeding risk, and treatment prediction
- No model is yet ready for routine clinical use — external multicentre validation is the critical next step
- ITP registries hold the data needed
- Need to start using registry data in AI & ML studies