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NEW INSIGHTS INTO IMMUNE THROMBOCYTOPENIA

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GENERATION OF SYNTHETIC PATIENTS IN IMMUNE THROMBOCYTOPENIA: EXPERIENCE FROM THE ITALIAN GIMEMA ITP0918 REGISTRY

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Disclosures of
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The Italian Registry on Active Adult ITP

The GIMEMA ITP 0918 Italian registry, sponsored by GIMEMA Foundation (Rome) and managed by the Hematology Project Foundation (Vicenza) with partial support from Novartis, **started in 2019** and, so far, has collected data on **1087 evaluable patients** across **29 Italian centers**.

The aim of the registry is to provide a dynamic picture of the **history and management** of ITP in Italy by collecting and analyzing standardized data.

Both **retrospective and prospective** data are collected, with patients followed for up to 5 years.

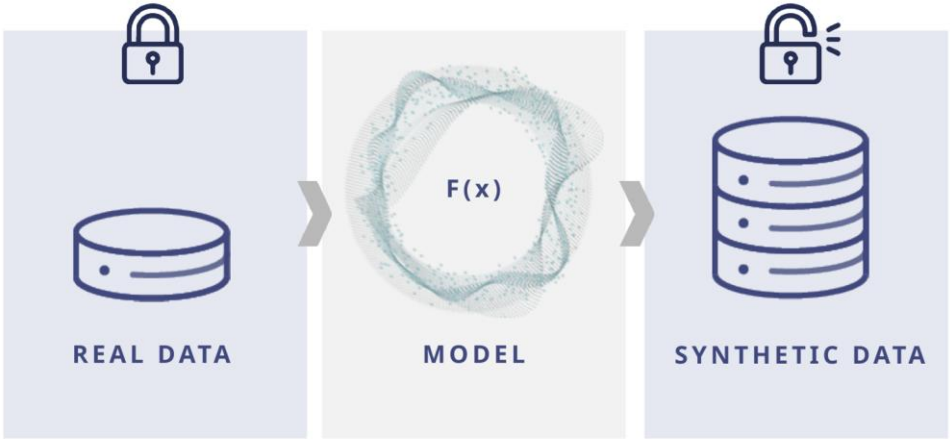
Why Synthetic Data?

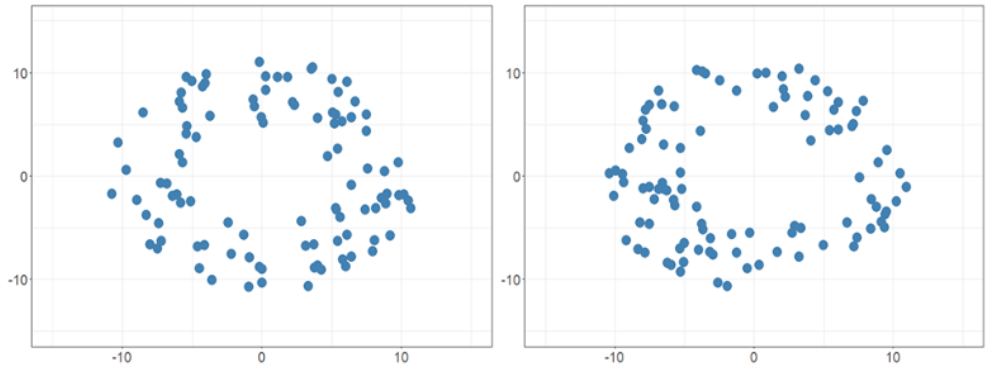
1	2	3	4
ANONYMIZATION	DEMOCRATIZE INNOVATION	OPTIMIZE DATA COLLECTION	DATA AUGMENTATION
STEP FORWARD IN CURRENT TECHNOLOGY	BROADER ACCESS TO DATA	MAKE IT CHEAPER AND MORE TIMELY, LOWER COSTS	

What Is Synthetic Data?

We used individual-level data from 1,087 patients in the registry to generate a synthetic cohort of 2000 records, covering demographic characteristics, clinical and laboratory data, bleeding history, treatments, and outcomes, for a total of 41 variables.

SYNTHESIS METHOD	OTHER OPTIONS
Sequential synthesis Synthpop R library [2] CART	GANs [1] Autoencoders Bayesian Networks …





[1] Goodfellow et al. (2014)
[2] Nowok et al. (2016)

Internal Consistency

Baseline conditions were classified as:

GROUP A: patients never treated before for whom a treatment is initiated for the first time.

GROUP B: patients already treated for whom a new line of ITP treatment is initiated.

GROUP C: patients under any treatment.

Assignment to treatments was controlled through a set of **logical constraints**.

	SYNTHETIC COHORT		
Treatment	**A** **N = 316[1]**	**B** **N = 369[1]**	**C** **N = 1,315[1]**
Corticosteroids	207 (66%)	93 (25%)	347 (26%)
HD Dexamethasone	113 (36%)	51 (14%)	47 (3.6%)
Immunoglobulin	85 (27%)	23 (6.2%)	33 (2.5%)
Eltrombopag	6 (1.9%)	148 (40%)	625 (48%)
Romiplostim	7 (2.2%)	45 (12%)	261 (20%)
Rituximab	5 (1.6%)	16 (4.3%)	9 (0.7%)
Fostamatinib	0 (0%)	13 (3.5%)	51 (3.9%)
Azathioprine	0 (0%)	10 (2.7%)	18 (1.4%)
Cyclosporin A	0 (0%)	17 (4.6%)	32 (2.4%)
Cyclophosphamide	0 (0%)	1 (0.3%)	4 (0.3%)
Dapsone	0 (0%)	4 (1.1%)	4 (0.3%)
Mycophenolate mofetil	0 (0%)	6 (1.6%)	19 (1.4%)
Tranexamic acid	0 (0%)	2 (0.5%)	7 (0.5%)
Platelet transfusion	7 (2.2%)	4 (1.1%)	6 (0.5%)
Other	0 (0%)	28 (7.6%)	51 (3.9%)

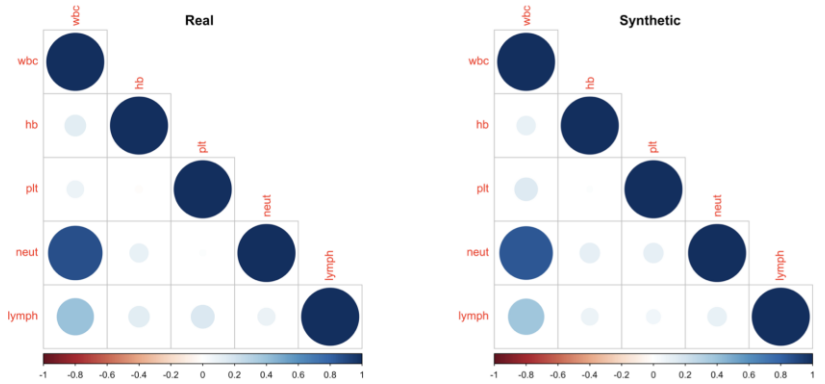
[1] n (%)

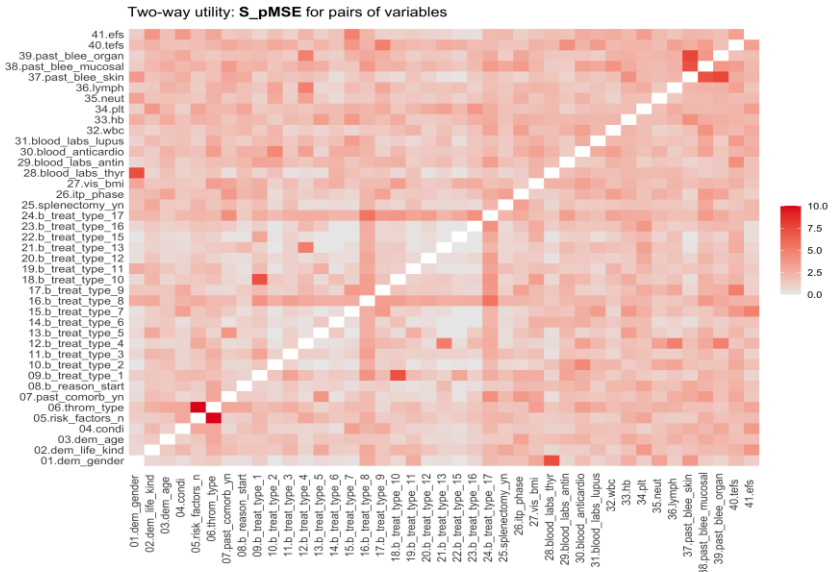
Data Fidelity

Characteristic	Real N = 1,087[1]	Synthetic N = 2,000[1]	p-value[2]
Gender			0.8
Male	455 (41.9%)	828 (41.4%)	
Female	632 (58.1%)	1,172 (58.6%)	
Age at diagnosis	52 (2, 92)	53 (2, 92)	0.7
Unknown	6	12	
Risk factors for thrombosis			
0	461 (42.6%)	822 (41.4%)	
1	302 (27.9%)	578 (29.1%)	
2	168 (15.5%)	323 (16.3%)	
3	96 (8.9%)	158 (8.0%)	
4	38 (3.5%)	73 (3.7%)	
5	15 (1.4%)	28 (1.4%)	
6	3 (0.3%)	4 (0.2%)	
Unknown	4	14	
Comorbidities related to ITP treatment (groups B /C)	67 (6.4%)	124 (6.4%)	>0.9
Unknown	46	77	
ITP phase			>0.9
Newly diagnosed	221 (20.4%)	399 (20.1%)	
Persistent	115 (10.6%)	220 (11.1%)	
Chronic	745 (68.9%)	1,363 (68.8%)	
Unknown	6	18	
White blood cells (x10^9/L)	7.3 (1.3, 30.6)	7.3 (1.3, 30.6)	0.8
Unknown	28	42	

[1]n (%); Median (Min, Max)

[2]Pearson's Chi-squared test; Wilcoxon rank sum test; Fisher's exact test

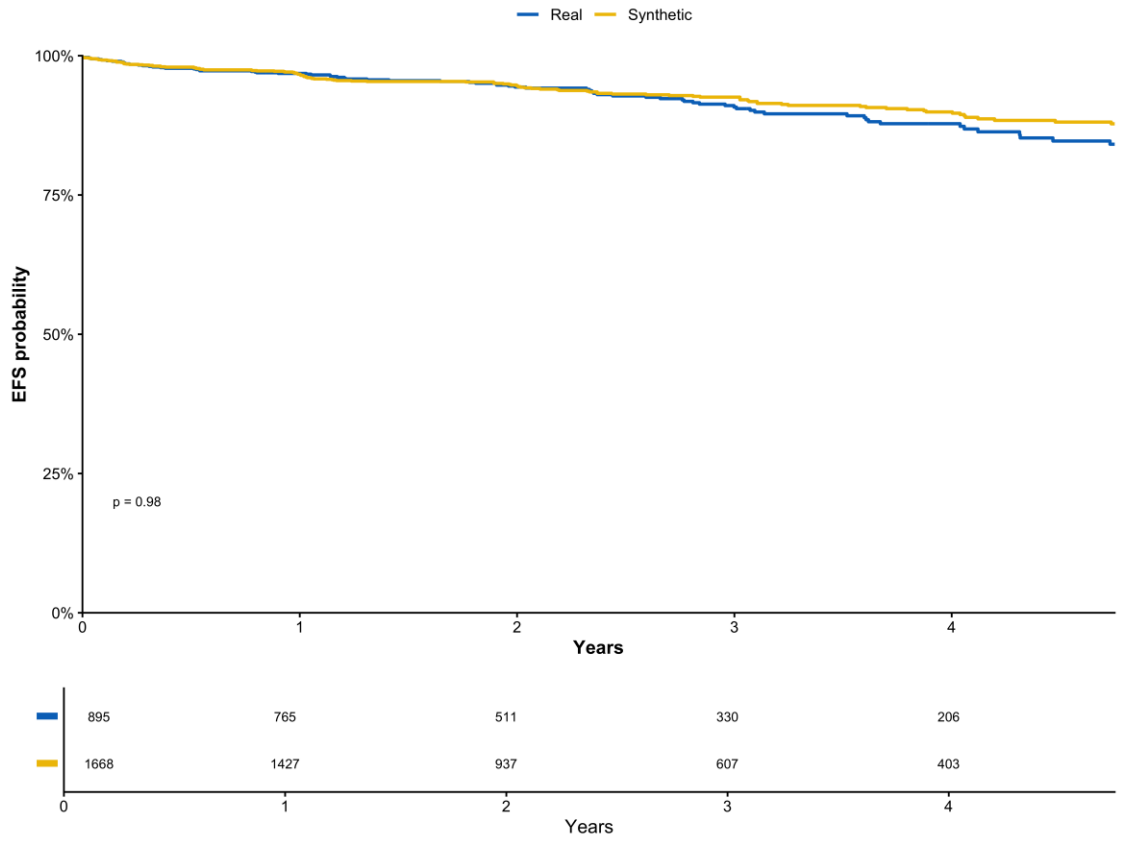




Survival & Privacy

Event-Free Survival (EFS) was defined as the time from the first visit to the occurrence of a thromboembolic event or death from any cause, whichever occurred first. The estimated survival curves are nearly **superimposable**, and the censoring process appears to be well replicated.

None of the real patients were copied in the synthetic dataset. Moreover, we obtained good results for the **NNDR (0.81)**, indicating adequate distance to real data.



Conclusions

We were able to generate a synthetic cohort that closely mimic the original data - What can we conclude?

Where they can help

a) Privacy-preserving data sharing
b) Simulation scenarios
c) Rare-disease and difficult-trial contexts

Core limits

a) Bias may be reinforced
b) Joint structure may be wrong
c) Privacy-fidelity trade-off

Next step: capture more complex structures within our registry, such as the longitudinal dimension of treatments and responses.

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