EHMA 2024

Shaping and managing innovative health ecosystems

Health Technology Assessment of METAglut1™ test for the diagnosis of GLUT1 deficiency within pediatric setting

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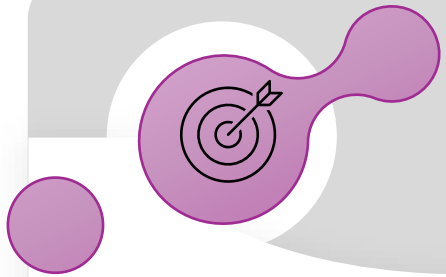
Relevance of the topic

- Glucose transporter type 1 deficiency syndrome (Glut1DS) is a **rare genetic metabolic disorder** characterized impaired glucose transport across the blood-brain barrier leading to neurological deficits
- The **rapid diagnosis is important to prevent Glut1DS complications**: the ketogenic diet is highly effective in controlling the seizures and improving gait disturbance and is generally well tolerated
- The standard diagnostic procedure is represented by the **lumbar puncture**, an invasive procedure
- However, **most patients required genetic analysis**, with long reporting times, to confirm the diagnosis
- In this specific setting, **METAglut1™ emerged as an innovative in vitro diagnostic test, supporting Glut1DS diagnosis, giving a response within 24-72 hours**
- Despite its strategic and clinical relevance, no **consensus exists in the Italian setting with regard its use in the clinical practice**

Objective



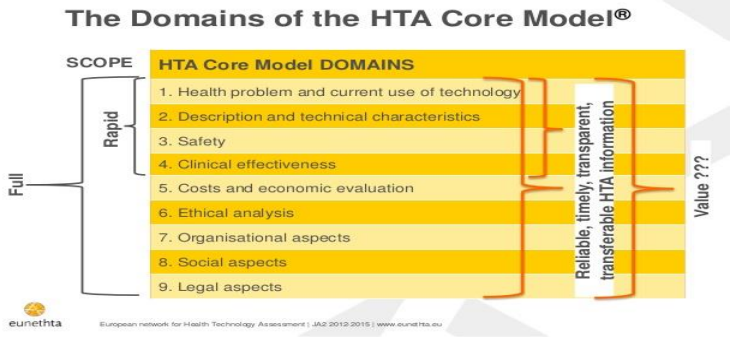
To define the multidimensional impacts related to the implementation of the innovative blood test for Glut1DS diagnosis, in comparison to the standard diagnostic tools used to date in the Italian clinical practice, thus producing real-life information defining the test economic and organizational sustainability and supporting the development of an adequate reimbursement tariff

The conduction of the HTA would be capable to answer to the following policy question: "***Which are the main benefits related to METAglut1™ for the diagnosis of Glut1DS, considering not only the hospital perspective, but also the clinical benefit for patients and potentially generalizable in the European context?"***

Methods

A Health Technology Assessment (HTA) analysis was performed, to bring together evidence and other relevant and reliable information for hospital managers to guide good investment decisions, within Glut1DS setting

HTA Core Model EUnetHTA



Scientific Evidence

Narrative literature review, *to define efficacy and safety indicators, concerning the comparison of the traditional and the innovative diagnostic tools*

Quantitative approach

Economic assessment *of the patient diagnostic clinical pathways, considering METAglut1™ presence or absence, and conduction of a* ***budget impact analysis*** *to define METAglut1™ economic and financial sustainability*

Qualitative approach

Administration of specific qualitative questionnaires and interviews to healthcare professionals*, examining their perceptions and acceptance in the conduction of the innovative diagnostic test*

Results from the narrative literature review

Safety

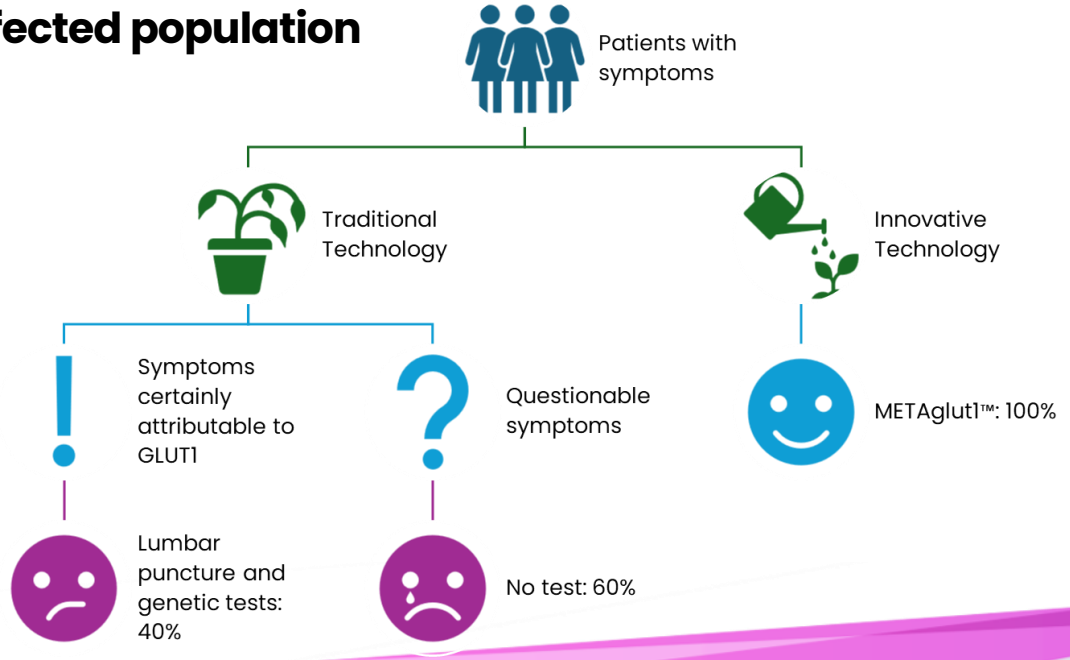
Technology			
Adverse events	**Lumbar Puncture**	**Genetic Test**	**METAglut1™**
Headache	18% (p<0.05) (Ebinger & Rating, 2004)	/	/
Back Pain	24% (p<0.001) (Ebinger & Rating, 2004)	/	/
Nausea	23% (Ebinger & Rating, 2004)	/	/

No impact on hospitals for the management of patients with the above adverse events

Efficacy

Literature declared that lumbar puncture and **METAglut1™ presents the same performances, in terms of sensibility and specificity**

The difference emerged in the proportion of patients that could be tested, In terms of **% coverage of potentially affected population**



Results from the economic analysis

Traditional situation: Absence of METAglut1™ *test*

The following 5 scenarios have been economically assessed and valorized:

1) Patient with symptoms that can certainly be attributed to GLUT1 DS and is tested with traditional technology and thus tests positive
2) Patient with symptoms that can certainly be attributed to GLUT1 DS and is tested with traditional technology and thus tests negative, but with an in-depth test (MLPA) in order to confirm or not the negativity
3) Patient with symptoms that can not certainly be attributed to GLUT1 DS, and is not tested
4) Patient with symptoms that is tested with the innovative technology, thus tests positive and is also tested with the traditional technology in order to confirm the positivity
5) Patient with symptoms that is tested with the innovative technology, thus tests negative

Innovative situation: Presence of METAglut1™ *test*

Economic assessment of the process

Time Horizon: 12 months

Traditional situation	Patient's Pathway	%*	Economic evalution
Tested patients	Patient's Pathway #1	35%	3,518.32 €
	Patient's Pathway #2	5%	4,063.02 €
Not tested patients	Patient's Pathway #3	60%	175.22 €
Average weighted cost related to the traditional situation			**1,539.70 €**

Innovative situation	Patient's Pathway	%*	Economic evaluation
Tested patients	Patient's Pathway #4	95%	5,286.63 €
	Patient's Pathway #5	5%	538.59 €
Average weighted cost related to the innovative situation			**5,049.23 €**

*Distribution of patients by pathway is derived from expert opinion based on the current and observed clinical practice

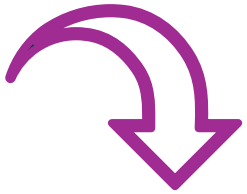
Economic assessment of the process

Time Horizon: 8 years

	%	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6	Year 7	Total Costs	Average weighted cost
Patient's Pathway #1	40%	3,518.32 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	4,569.66 €	Traditional situation: Absence of METAglut1™ test **4,596.90 €**
Patient's Pathway #2	5%	4,063.02 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	5,114.37 €	
Patient's Pathway #3	60%	175.22 €	175.22 €	175.22 €	3,518.32 €	175.22 €	175.22 €	175.22 €	4,569.66 €	
Patient's Pathway #4	95%	5,286.63 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	6,337.98 €	Innovative situation: Presence of METAglut1™ test **6,100.58 €**
Patient's Pathway #5	5%	538.59 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	175.22 €	1,589.93 €	

Budget Impact Analysis

	Traditional Situation	Innovative Situation
Baseline Scenario	100%	0%
Scenario 1	80%	20%
Scenario 2	50%	50%
Scenario 3	20%	80%
Scenario 4	0%	100%



	Total costs	Difference (Euro)	Difference (%)
Baseline Scenario	307,939.52 €	0.00 €	0.0%
Scenario 1	362,856.09 €	54,916.56 €	17.8%
Scenario 2	445,230.94 €	137,291.41 €	44.6%
Scenario 3	527,605.78 €	219,666.26 €	71.3%
Scenario 4	582,522.35 €	274,582.82 €	89.2%

From the economic assessment of the hospital costs to the economic assessment of the social costs

	Patient's Pathway #1	Patient's Pathway #2	Patient's Pathway #3	Patient's Pathway #4	Patient's Pathway #5
% tested patients	35%	5%	60%	95%	5%
Number of Hospital Accessed	5	6	2	6	4
Productivity loss related to the hospital accesses	425.00 €	510.00 €	170.00 €	510.00 €	340.00 €
Productivity loss related to the lumbar puncture	40.80 €	40.80 €		40.80 €	
Trasportation	75.00 €	90.00 €	30.00 €	90.00 €	60.00 €
Drug (Levetiracetam doc 500 mg)		22.60 €*	452.04 €		452.04 €
Cognitive and behavioral support		90.00 €*	1,800.00 €		1,800.00 €
Social cost by patient's pathway	**540.80 €**	**753.40 €**	**2,452.04 €**	**640.80 €**	**2,652.04 €**
Average weighted social cost		**1,698.17 €**		**741.36 €**	

- 56%

Results from the healthcare professionals' perceptions

Dimensions	Standard Technology	Innovative Technology	
Safety	0.39	0.46	❑ No difference on the development of adverse events related to the procedure ❑ METAglut1™ test is well tolerated
Effectiveness	-0,25	1.33	❑ Higher and prompt Glut1DS detection rate
Equity impact	-0.17	-0.75	❑ Poor access to care on local level, due to the test's scarce availability ❑ Potential generation of health migrations phenomena
Social impact	-0.08	0.92	❑ Improved patients' and families' satisfaction ❑ Improved patients' quality of life ❑ Reduced social costs due to the diagnostic pathways
Legal impact	1.13	1.00	❑ Need to regulate the acquisition of METAglut1™ test
Organizational impact	0.50	-1.25	❑ In the short term, training courses and hospital meetings are required for all the healthcare professionals involved, ❑ An improvement in the patient's clinical pathway may emerge

Conclusions

Results revealed **the potentialities of METAglut1™ in the improvement of the diagnostic pathway of such rare disease**

Healthcare **professionals recognized benefits of METAglut1™ within all the HTA domains,** thus declaring that its routinary use would optimize the overall patient management

Despite the need of additional investment that is absorbed in the long run given METAglut1™ capability to modify the patients' pathway thanks to an accurate and prompt diagnosis, **a higher clinical benefit emerged with a consequent positive impact on the social point of view**

A real-life data collection would be required to make the results more robust and scalable, thus also considering a long-term time-horizon

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Thank you

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