Investor & Media presentation, Paris - March 15, 2011

2010 annual results

exonhit therapeutics

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AclarusDx® CE

- First ExonHit diagnostic test to have CE marking
- New chapter in the history of ExonHit
- Beginning of a marketing activity in addition to current R&D activities

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Speakers

- Loïc Maurel, M.D., President of the Management Board
- Hervé Duchesne de Lamotte, Chief Financial Officer and Member of the Board
- Matthew Pando, Ph.D., Executive Vice President, Therapeutics and Member of the Board
- Isabelle Barber, Senior Vice President, Global Marketing, Diagnostics

Agenda

- **Introduction**
- 2010 key events and 2011 outlook
- 2010 financial results
- Therapeutics
- Diagnostics
- Conclusion
- Questions & Answers

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ExonHit Therapeutics today

- **Established business**
  - Founded in 1997. Listed on Alternext since 2005 (ALEHT)
  - HQ in Paris. US subsidiary in Maryland
  - Employs 66 associates

- **Unique know-how in Therapeutics and Diagnostics**
  - Two domains: Cancer and Alzheimer's disease
  - A recognized and validated technology & expertise in genomics
  - Collaborations with Allergan, bioMérieux, Institut Gustave Roussy

- **A solid intellectual property**
  - 127 patents and 264 in the world

- **Academic and industrial partners**

ALLERGAN

BIOMÉRIEUX

Institut de cancérologie GUSTAVE ROUSSY VILLEJUIF - www.igr.fr

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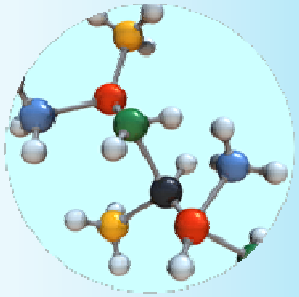
ExonHit assets in personalized medicine

GWSA

Therapeutics

Diagnostics

New molecules



Diagnostic tests

Personalized medicine

Biomarkers & Companion diagnostics

GWSA: Genome-Wide SpliceArray™

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2010 key events

EHT/AGN 0001	**Sub-licensed to Bristol-Myers Squibb by Allergan**
EHT/AGN 0003	**Inclusion into Allergan collaboration**
EHT 0202	**Identification of a blood-based transcriptomic signature**
AclarusDx®	**Completion of key steps for CE marking** **R&D grant from the US Congress (also for EHT 107 program)**
EHT Dx14	**Patient recruitment for the validation study**
RedPath	**Cancellation of the planned US acquisition**

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History of the planned acquisition of RedPath

Date	Event
April 25, 2010	Signature of a merger agreement for the acquisition of RedPath
June 18, 2010	Announcement of a potential discontinuation of coverage by Highmark* for PathFinderTG®
June 28, 2010	Extraordinary shareholders' meeting on 1st notice
July 8, 2010	Extraordinary shareholders' meeting on 2nd notice Submission by RedPath of a file supporting maintenance of coverage for PathFinderTG®
September 20, 2010	Highmark's decision to limit reimbursement
October 25, 2010	Cancellation of the acquisition

**Medicare is the US federal health insurance agency for elderly people*

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2010 financial results

Consolidated income statement Thousand €	**2010**	2009
Total revenues	**8 418°°**	**4 892**
Research and development expenses	**(8 480)**	(8 984)
Marketing and sales expenses	**(1 334)**	(1 239)
General and administrative expenses	**(5 578)**	(4 329)
Total operating expenses	**(15 392)**	**(14 552)**
Loss from operation	**(6 974)**	**(9 659)**
Interest expenses	**(2 230)***	(277)
Interest income	**271**	690
Net exchange gain (loss)	**(144)**	(70)
Tax benefit	**1 329**	1 616
Net income (loss)	**(7 748)**	**(7 701)**
Net profit (loss) per share (€)	(0.23)	(0.27)

* Impact of the cancellation of the planned RedPath acquisition: 1.1M€ fees (2009/10)
+ 0.7M€ loan to RedPath

°° Non-recurring payment of $4M (~3€M) by Allergan (deal with BMS)

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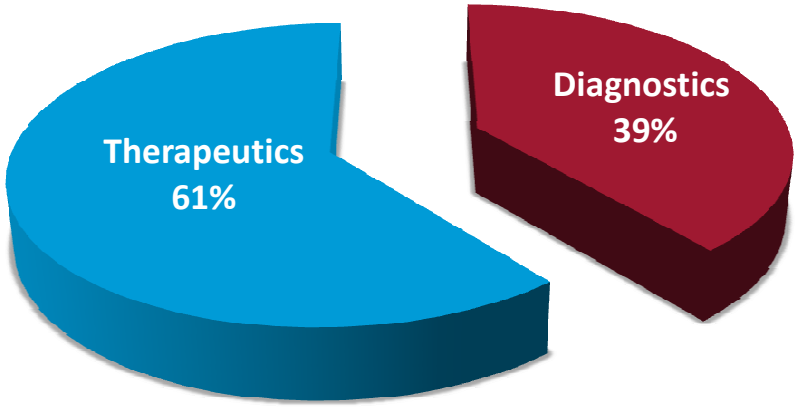
R&D investments in Therapeutics and Diagnostics

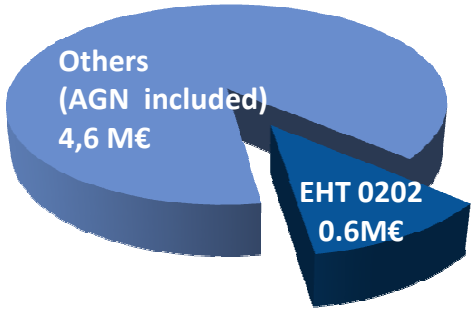
Consolidated R&D expenses

(8.5 M€)



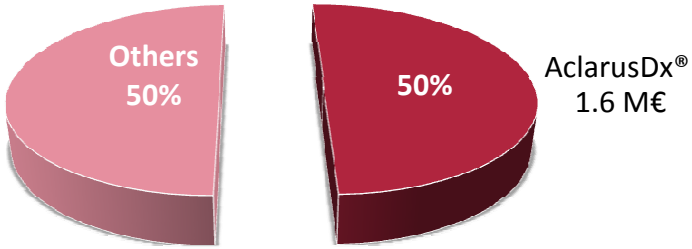
Therapeutics

(5.2 M€)



Diagnostics

(3.3 M€)



A cash position amounting to € 25.6 millions

Consolidated balance sheet Thousand €	**31.12.2010**	**31.12.2009**
Total long-term assets	1 772	2 307
Short-term assets	3 883	3 996
Cash and cash equivalents	**25 607**	**30 245**
Total Assets	**31 261**	**36 549**
Shareholder's equity	19 191°°	25 458
Convertible bonds	6 522	6 522
Provisions for risks*	1 534	344
Total long-term liabilities	15	200
Total short-term liabilities	3 999	4 024
Total liabilities and shareholders' equity	**31 261**	**36 549**

* Includes €1,3M accrual for reimbursement of convertible bonds

°° TEPA capital increase

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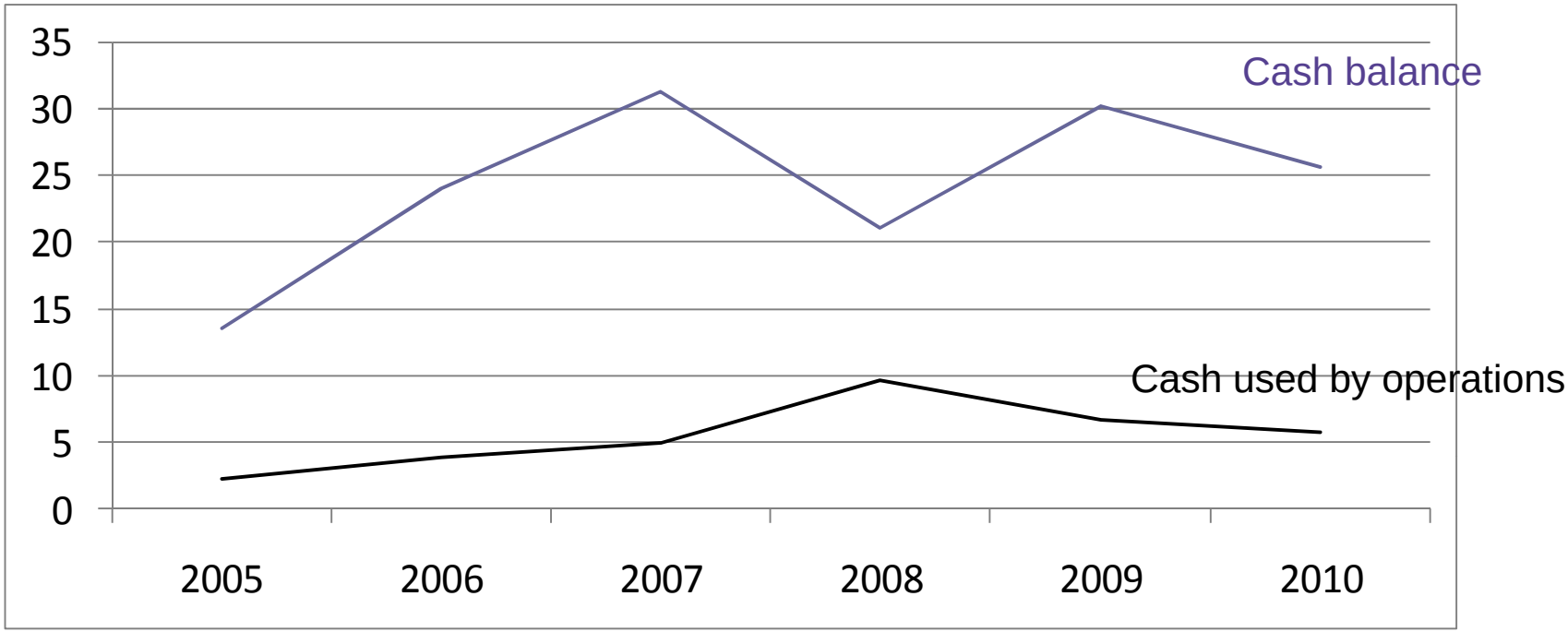
A healthy financial situation



Années	2005	2006	2007	2008	2009	2010
Cash balance (M€)	13.6	24.0	31.3	21.1	30.2	25.6
Cash used by operations (M€)	2.2	3.9	4.9	9.6	6.7	5.8

Net cash used by operations planned in 2011: 9-11 M€

2011: investing for the introduction of AclarusDx®

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Convertible bonds

Gross amount of the initital issue	€ 13.5 million
Issue date	November 8, 2006
Issue price	€ 6.50 €
Number of convertible bonds initially issued	2,080,335
Number of outstanding bonds (as of Dec. 31, 2010)	1,003,412
Annual interest rate	3.50%
Redemption at maturity	Bonds mature on November 8, 2011 and will be redeemed at €7.75/bond with reimbursement premium of €1.25/bond

As of June 30 2010, accrual of 1.3 M€ corresponding to the potential reimbursement premium of ExonHit convertible bonds

2011 goal: refinancing by issuance of new convertible bonds

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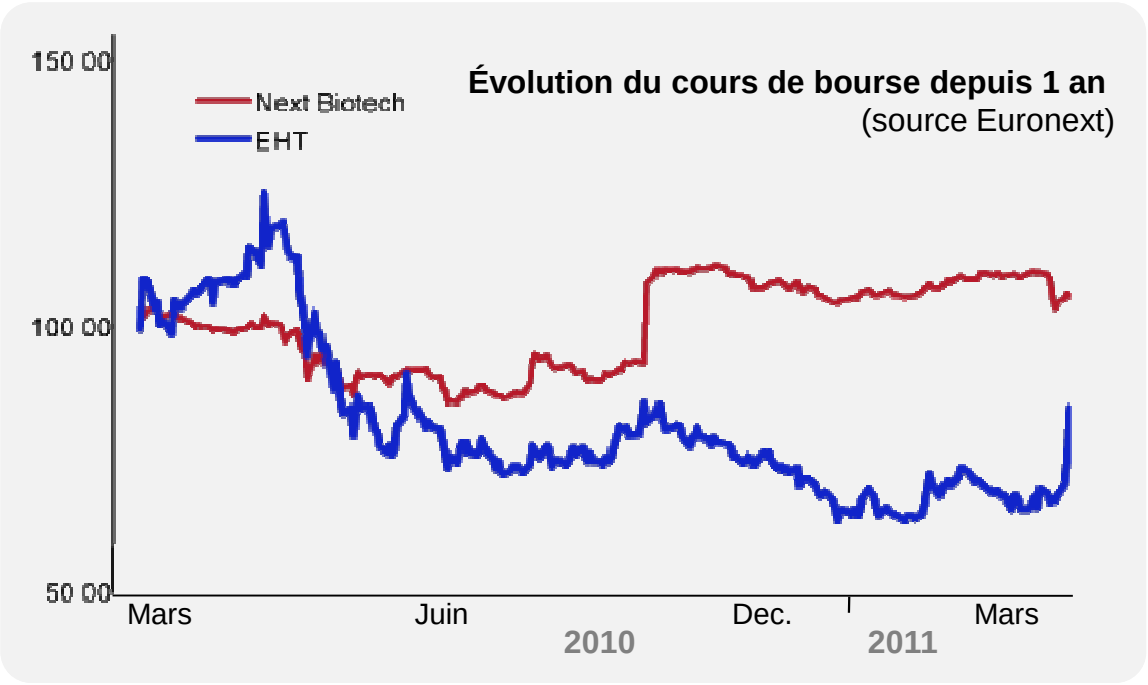
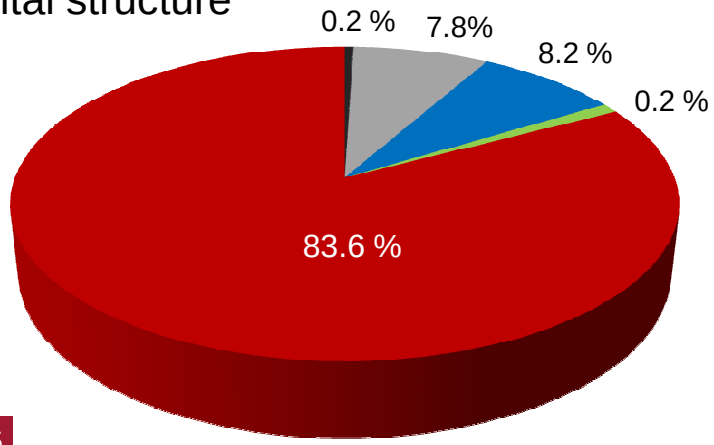
ExonHit share price (Alternext : FR0004054427 – ALEHT)

- 1 year high: 4.15 €
- 1 year low: 1.95 €
- Share price on 14/03/11: 2.40 €
- Market capitalization ou 14/03/11: 79.93 M€
- Average liquidity (12 months): 132,000 shares /day



Capital structure*



- **Mandataires sociaux**
- **Groupe Oxford Bio Science**
- **Institutionnels**
- **Autocontrôle**
- **Public**

* On the basis of a Bearer Securities Identification (TPI) dated February 16, 2011

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Our strategy in Therapeutics

- **Continuation of the collaboration with Allergan**

- **Partner programs as early as possible**
  - EHT 0202
  - EHT 107 Program

- **Start new programs only in collaboration**
  - R&D collaborations
  - Partnerships with license rights

- **Extension of the scope of partnerships**
  - Association of biomarkers and companion diagnostics if appropriate

Moving to a pure partnering model to limit risk exposure

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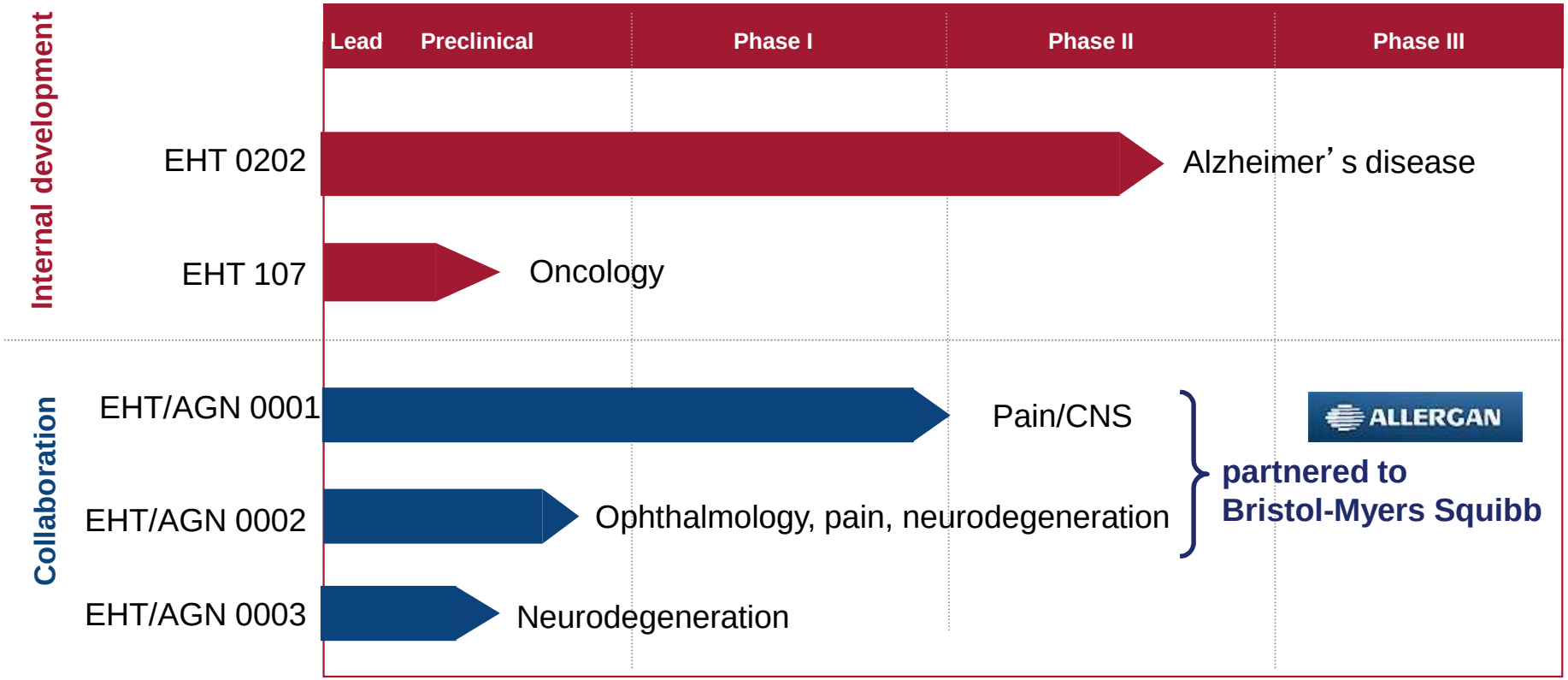
Therapeutic pipeline



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The Allergan-ExonHit collaboration: A success story

ALLERGAN

Partnership since 2002

Extended 3 times

Collaboration in neurology, pain, ophthalmology

EHT/AGN 0001 out-licensed to BMS for $410 M deal in March 2010

Upfront payment of $4M

EHT/AGN 0003

New program added in Q1 2010

Developing custom GWSA*

"We are happy that our collaboration with ExonHit has led to a successful clinical program and to have BMS committed to developing this novel therapy for neuropathic pain. We look forward to continuing our work with ExonHit and to discovering and developing additional promising compounds."

Dr. Scott Whitcup,
EVP of R&D, Allergan,
March 3, 2010

*GWSA : Genome-Wide SpliceArray™

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Complementarity between GWSA™ & emerging technologies

- Enriching **GWSA™ content** thanks to the information generated by **next-generation sequencing**

- **GWSA™ platform advantages:**
  - **Mature R&D platform**
  - **IVD certified**

Optimal product development and approval time-lines with a tool staying at the cutting edge

Ongoing efforts to pursue EHT 0202 clinical development

- **Search for a partner** to develop/co-develop/co-finance Phase IIb supported by an external firm

- **Increased awareness of EHT 0202 in Alzheimer's disease scientific community**
  - Phase IIa results accepted for publication in ***Current Alzheimer Research***

- Identification of a potential **blood-based transcriptomic signature specific to treatment response to EHT 0202** ("companion diagnostic")

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Our strategy in Diagnostics

- **Commercial launch of produts currently in the portfolio:**

EU → France, EU Extension

- Independent reference lab

US

- CLIA lab (LDT)

and/or

- IVD test (FDA)

- **Evolution of the portfolio:**
  - Collaborations with university hospitals
  - Focus on oncology
  - Predictive and prognostic tests

- **Evaluation of several platforms for long-term commercialization**

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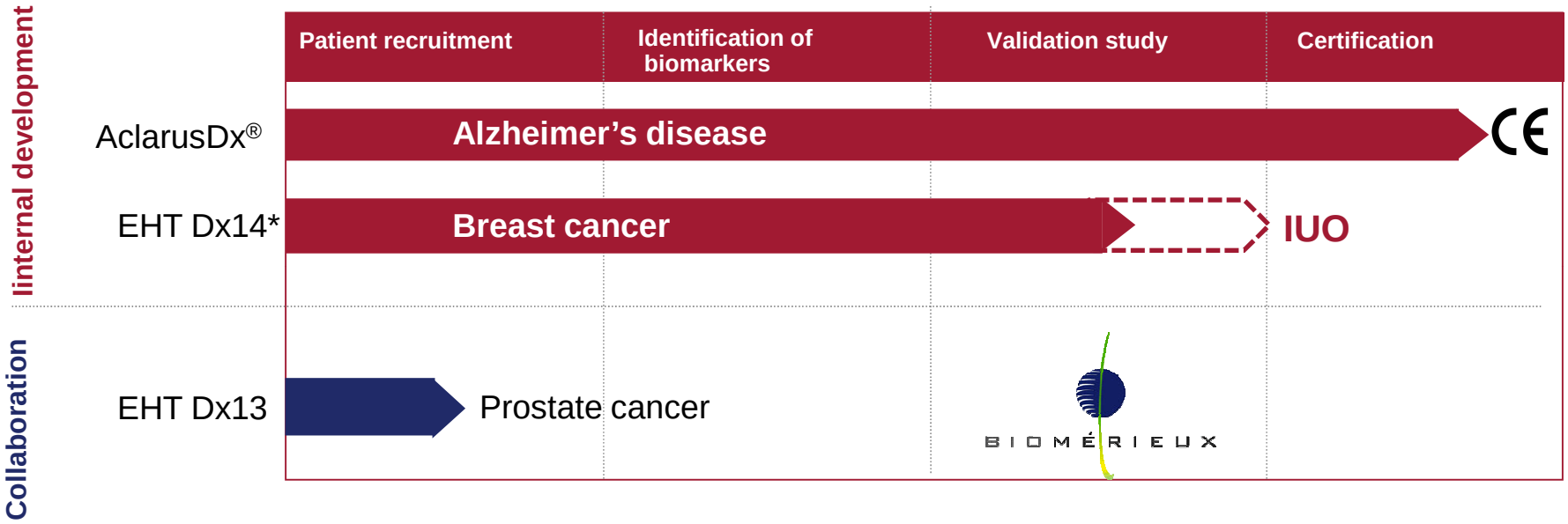
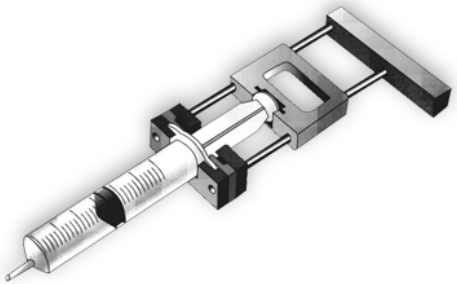
Diagnostic portfolio



*Tissue-based diagnostic

EHT Dx14 in breast cancer



- Breast cancer molecular signature developed with IGR

- EHT Dx14 + FNA*: improvement and standardization of FNA performances
  - + Performances equivalent to a biopsy
  - + Reducing use of invasive procedures
  - + Reducing time to results for the patients

- Introduction in major cancer centers in 2H 2011 as an Investigational Use Only product

*Fine Needle Aspiration

Institut de cancérologie GUSTAVE ROUSSY VILLEJUIF - www.igr.fr

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EHT Dx14: validation study is ongoing

- **Samples coming from Institut Gustave Roussy biobank**

- **Validation made in two steps:**

1) Confirmation of the performances published in *Lancet Oncology**
   - on 47 positive samples and 47 negative samples

➡ Results expected end of March

2) Demonstration of the added value of the test
   - on ≥ 62 inconclusive samples

➡ Results expected in July

*André et al. Exonic expression profiling of breast cancer and benign lesions: a retrospective analysis. *Lancet Oncology* 2009; 10:381-90

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AclarusDx®

A blood-based test intended to help in the diagnosis of Alzheimer's disease

Conformity letter sent to AFSSAPS

CE

EC Declaration of Conformity

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tel 33 (0)1 53 94 77 00
33 (0)1 58 05 47 19 **fax**

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75013 PARIS

MANUFACTURER:	ExonHit Therapeutics, SA
ADDRESS:	65, boulevard Massena, 75013 PARIS
EUROPEAN AUTHORIZED REPRESENTATIVE:	NA
PRODUCT(S) NAME(S):	AclarusDx™ set de prélèvement AclarusDx™ Assay Software Module AclarusDx™ Analysis Software AclarusDx™ Process Qualification Controls
CLASSIFICATION:	☐ ANNEX II-A ☐ ANNEX II-B ☐ DEVICE FOR SELF TESTING ☒ OTHER DEVICE
CONFORMITY ROUTE	☒ ANNEX III ☐ ANNEX IV.3 Full Quality System ☐ ANNEX IV.4 Product Design Examination ☐ ANNEX V Type Examination ☐ ANNEX VII Production Quality System
NEW PRODUCT(S)	☒ YES ☐ NO
GENERIC DEVICE GROUP CODE: (GMDN Nomenclature)	AclarusDx™ set de prélèvement : CT306 AclarusDx™ Assay Software Module : CT944 AclarusDx™ Analysis Software: CT1250 AclarusDx™ Process Qualification Controls: CT1649
APPLICABLE DIRECTIVE:	Directive 98/79/EC of the European Parliament and of the Council of 27 October 1998 on *in vitro* Diagnostic medical devices

SIGNATURE

Function	Associate Director, Quality Assurance and Project Management
Name	Caroline BOURGUIGNON
Signature	
Function	Chief Executive Office, Président du Directoire
Name	Loïc MAUREL
Signature	

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CE marking components

			ALMAC ISO 17025 ACCREDITED LABORATORY
Collection set		**Analysis software**	**Reference lab**

AclarusDx® CE

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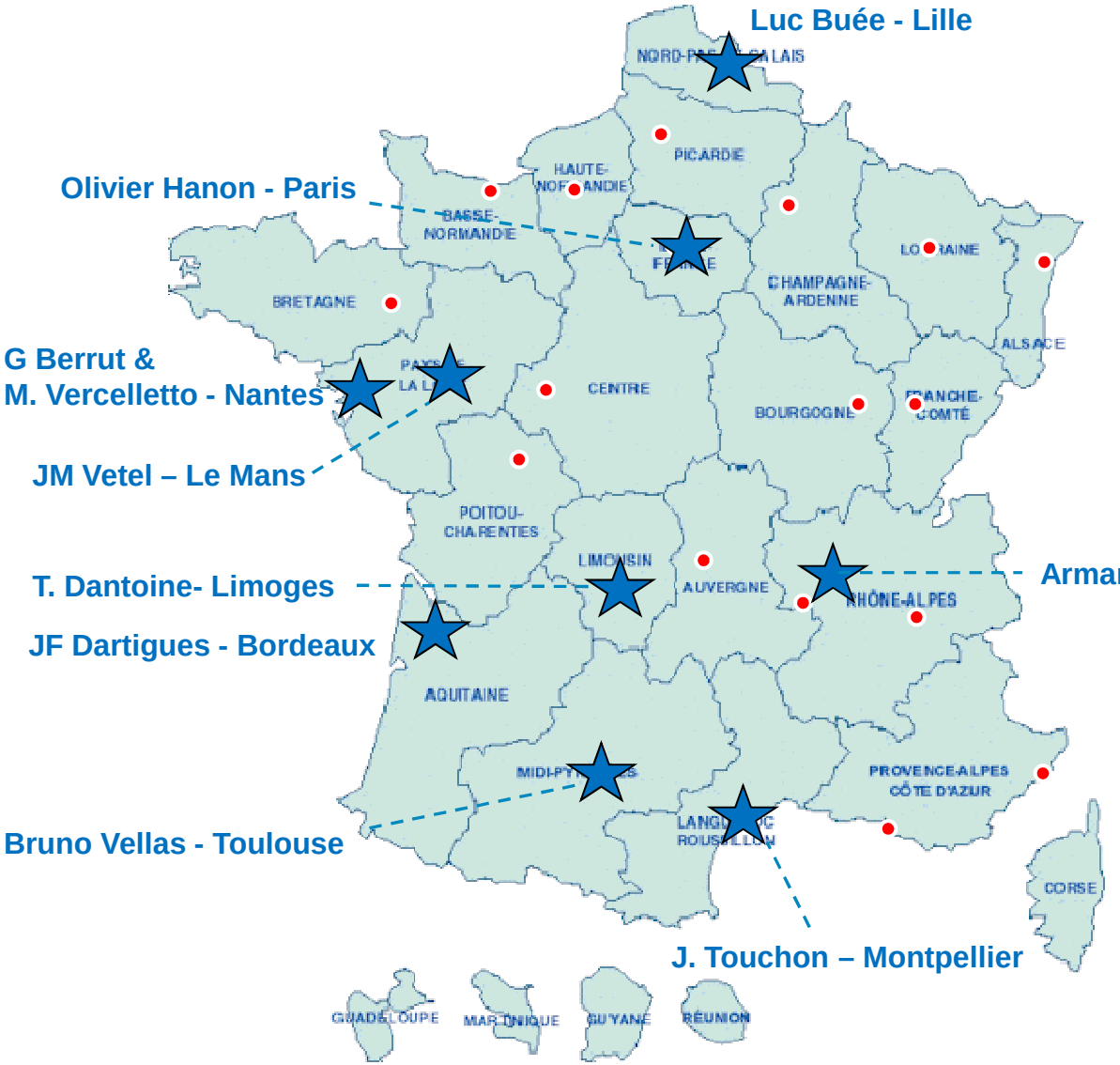
Organization of diagnostic structures in France



Split of hospital structures:

- 25 expert memory centers (CMRR)
- 300 memory centers networked to expert memory centers

★ Memory centers involved in AclarusDx® Scientific Board or having participated to the validation study

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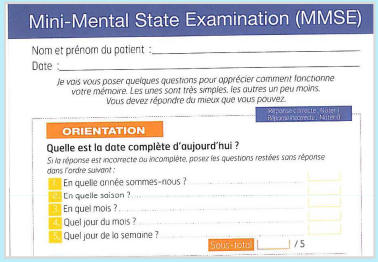
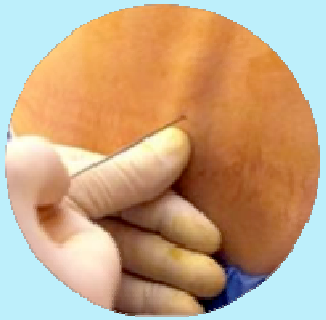
Today’s diagnosis of Alzheimer’s disease

Clinical assessment



Neuro-psychological tests

Imaging



Lumbar puncture

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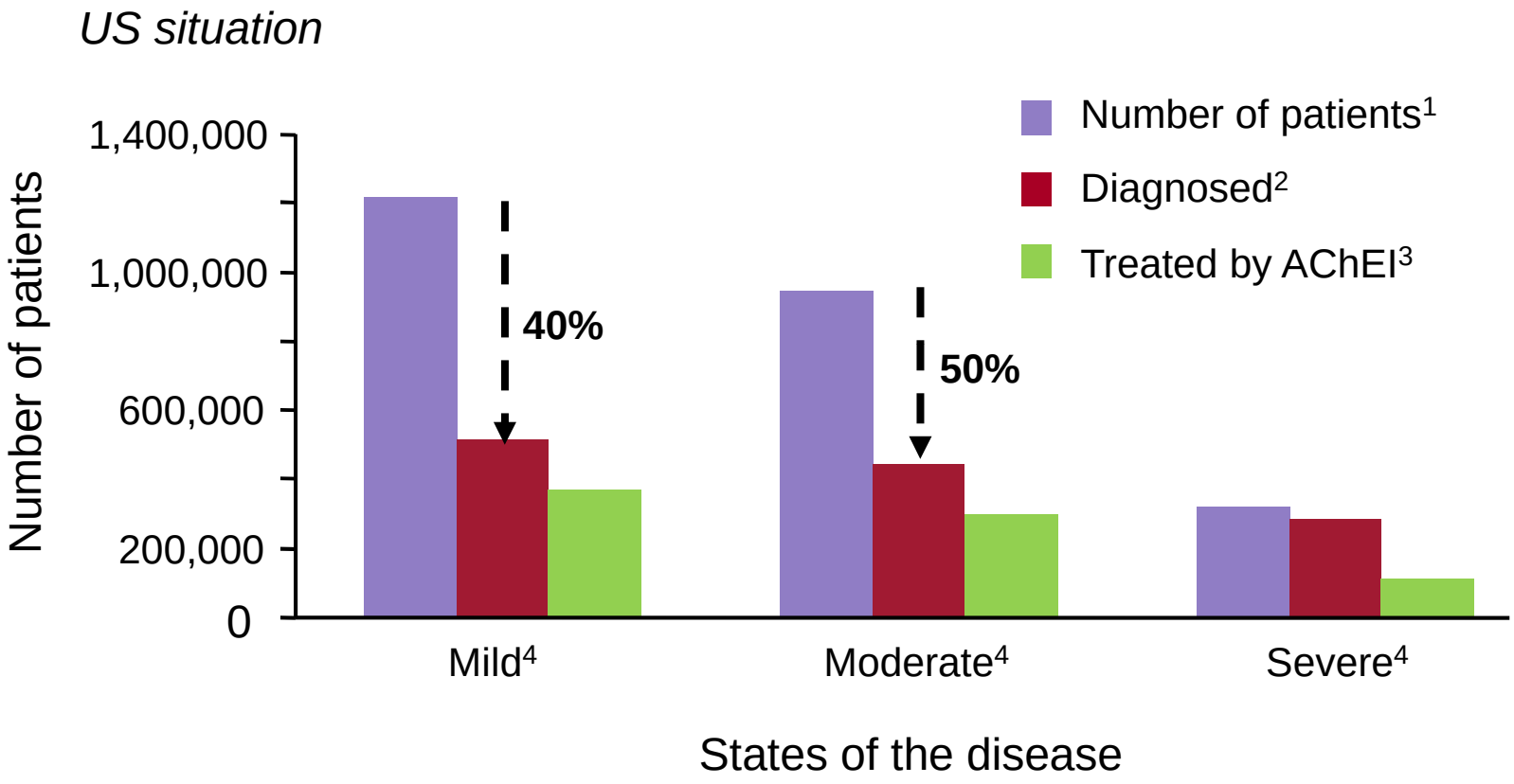
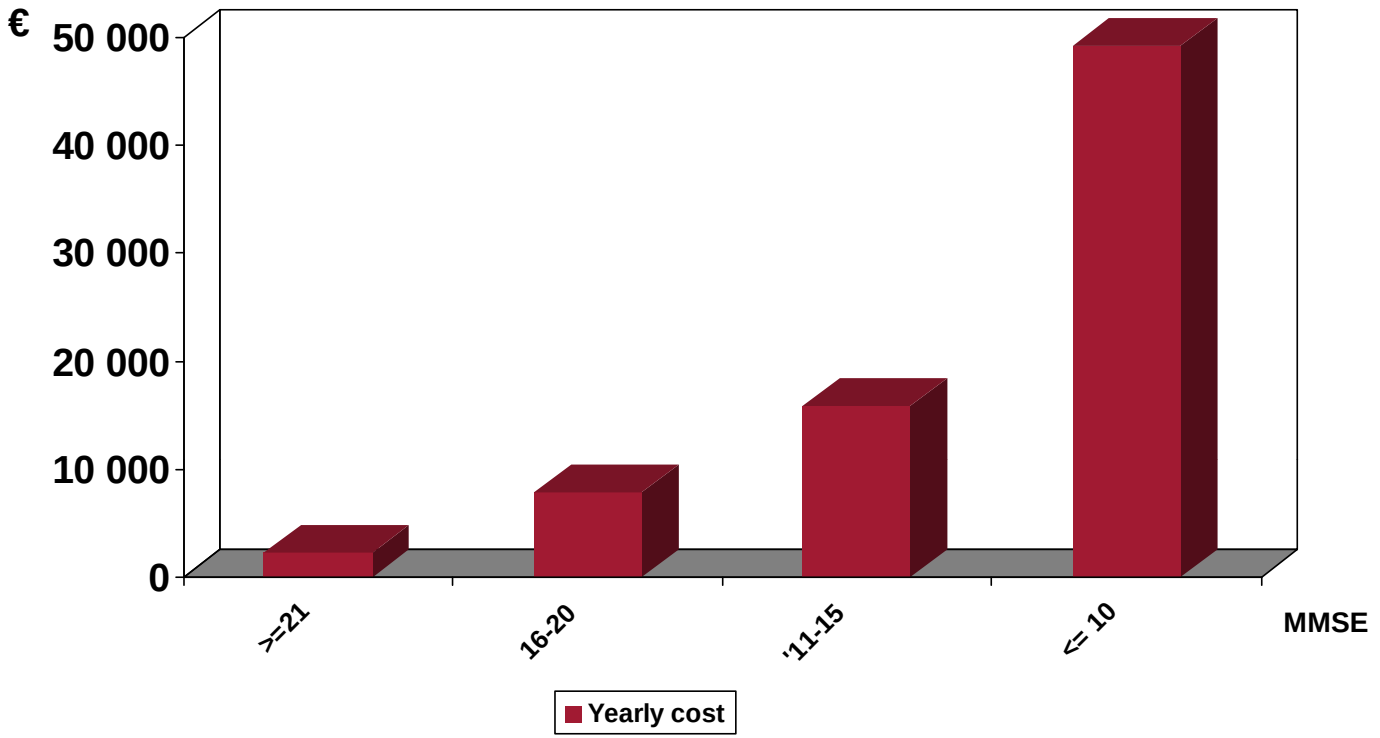
A diagnosis which is made too late…



AChE = acetylcholinesterase

[1]Mattson Jack; [2]MMI MDAD, 2001; [3]On CHeI—midpoint of last year's treatment by severity rates and rates reported in Reminyl uptake, 2001; [4]Decision Resources.

…and which impacts the cost of care



≥ 21 **MMSE score** **≤10**

+ **Disease progression** **+++++**

MMSE : Mini Mental State Examination

Rigaud AS et al. Patients with Alzheimer's disease living at home in France: costs and consequences of the disease. *J Geriatr Psychiatry Neurol*. 2003 Sep;16(3):140-5.

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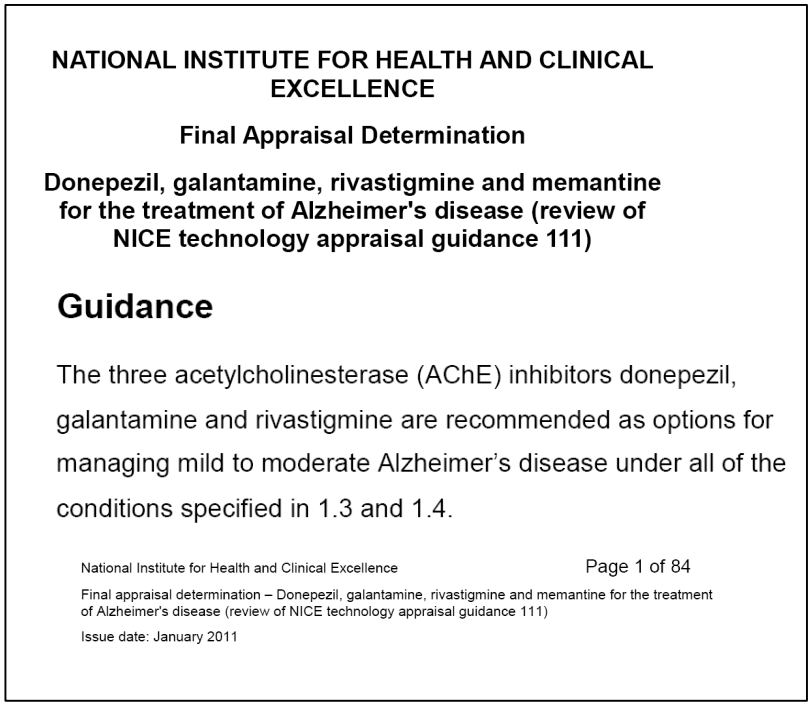
Interest of an early diagnosis



NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Final Appraisal Determination

Donepezil, galantamine, rivastigmine and memantine for the treatment of Alzheimer's disease (review of NICE technology appraisal guidance 111)

Guidance

The three acetylcholinesterase (AChE) inhibitors donepezil, galantamine and rivastigmine are recommended as options for managing mild to moderate Alzheimer's disease under all of the conditions specified in 1.3 and 1.4.

National Institute for Health and Clinical Excellence Page 1 of 84

Final appraisal determination – Donepezil, galantamine, rivastigmine and memantine for the treatment of Alzheimer's disease (review of NICE technology appraisal guidance 111)

Issue date: January 2011

NICE decision on January 18, 2011

Early care → delay of dependancy

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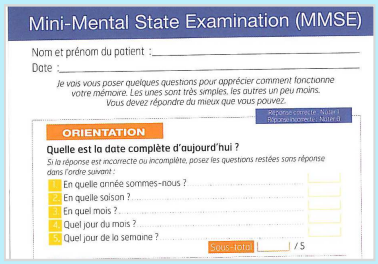
Tomorrow’s diagnosis of Alzheimer’s disease

Clinical assessment



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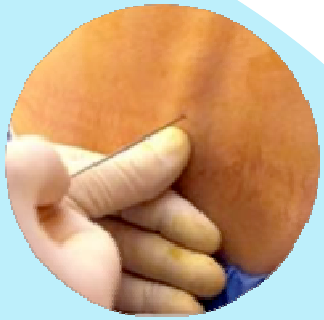

Lumbar puncture

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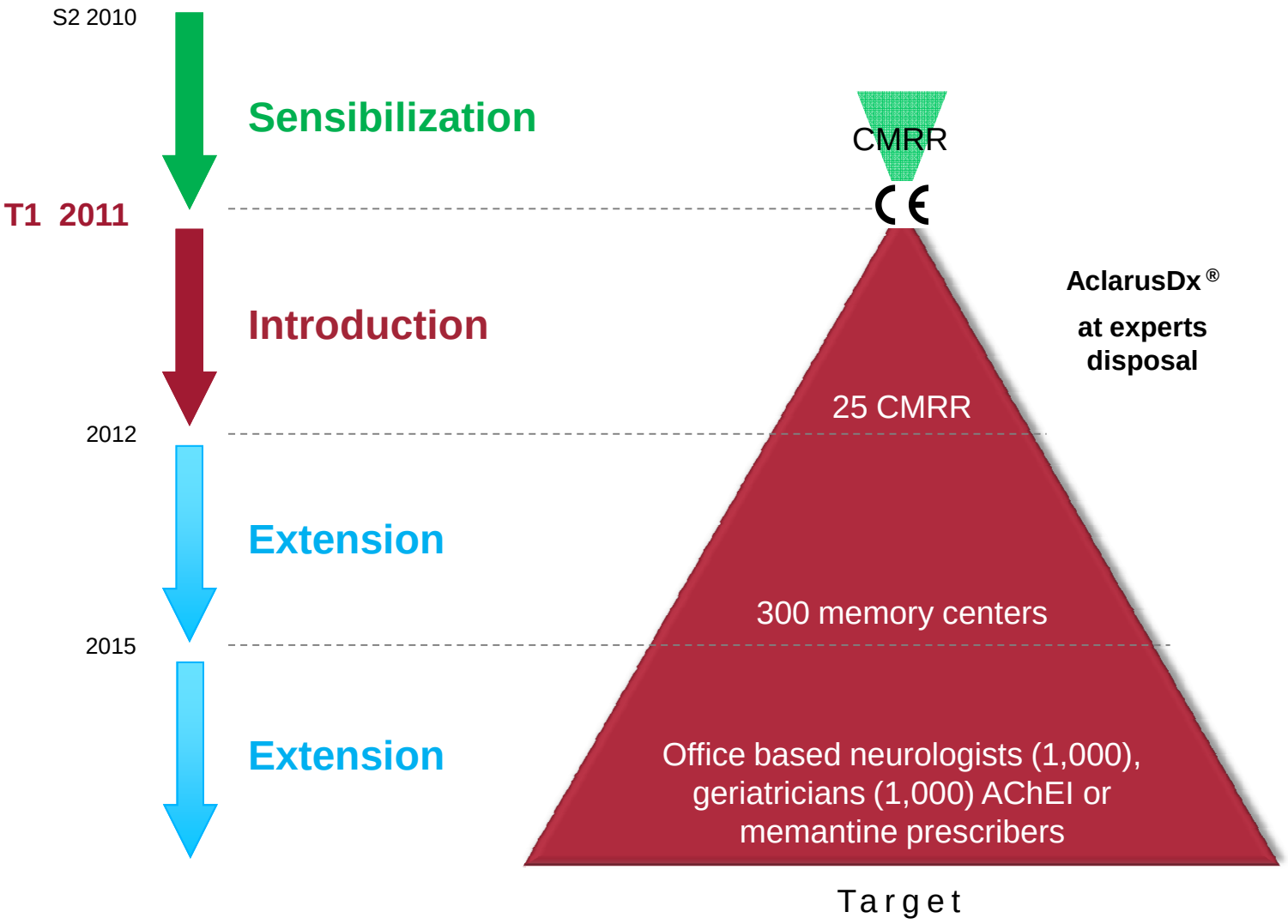
A sequential commercial introduction in France



…supported by a study in a real clinical setting

- **Main objective**: define AclarusDx® clinical value in expert memory centers real clinical setting

- **Cross-sectional, non interventional study:**
  - AclarusDx® is added to usual diagnostic exams

- **600 *de novo* patients**
  - Panel ≥ 15 CMRR

- **Total study duration: 18 months.** Inclusion of first patients: Sept. 2011

The main expert memory centers receiving directly new patients

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Conclusion

- AclarusDx® is CE marked allowing IVD use
- AclarusDx® will be introduced in a progressive manner in the French market
- AclarusDx® will be included into a study in a real clinical setting

ExonHit is entering into a new phase of its history

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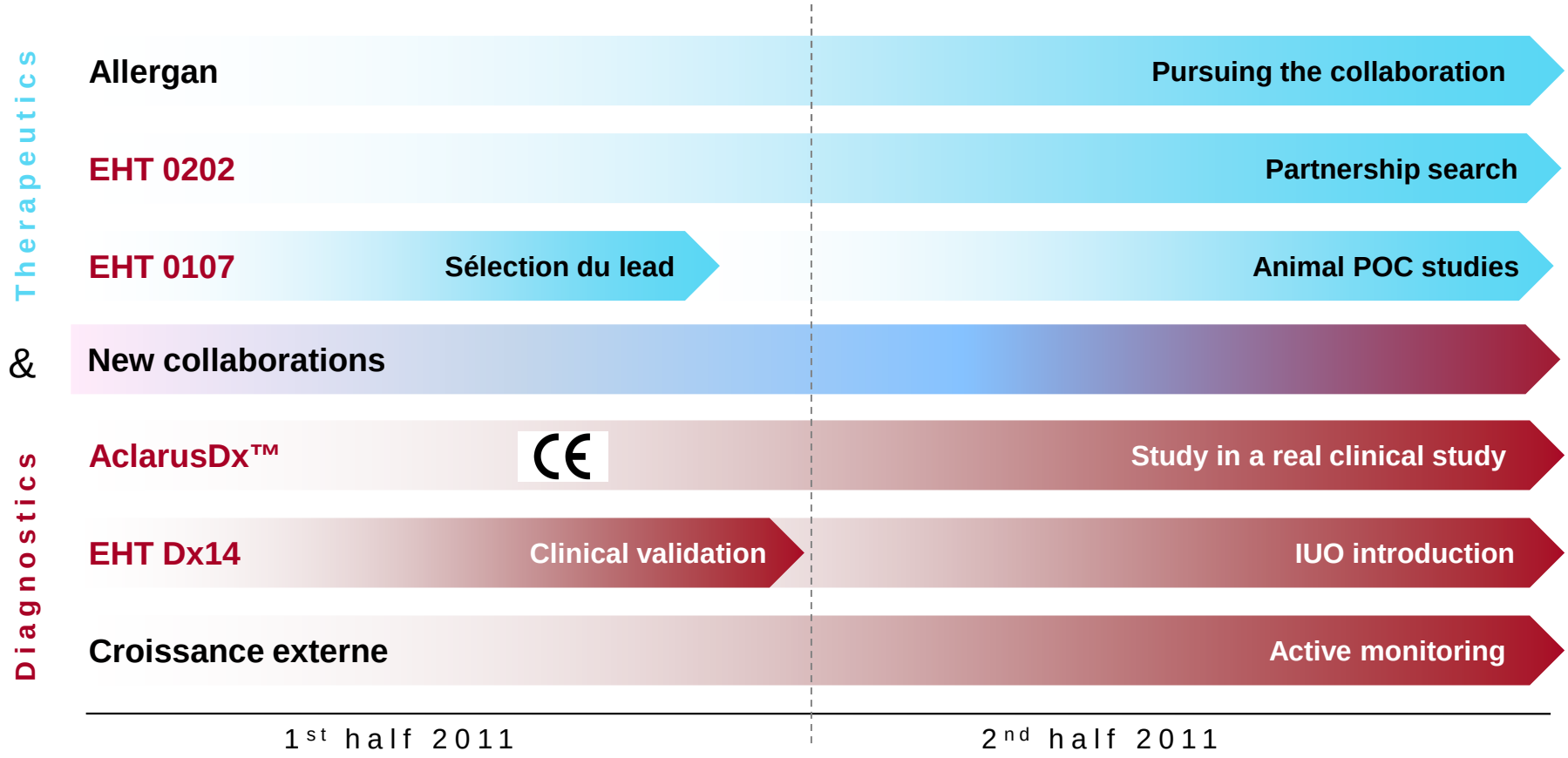
Conclusion

- Transformation into a company that starts to market products
- Continuing current partnerships and securing new collaborations
- Evolution towards personalized medicine thanks to the development of biomarkers and companion diagnostics

2011: focus on execution



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