



Karen Aiach, MSc./MBA

Case study:

Lysogene, A Leading Gene Therapy Company
initiated by Alliance Sanfilippo, seeded by private funders and
patient groups, chaired by a RD mother

6th Workshop of the Council of European Federations



October 30, 2013

FOUNDER

- Karen AIACH, M.Sc. MBA (ESSEC) – Research Degree in Economics (Paris 8)
 - ✓ Founding president and CEO of LYSOGENE
 - ✓ With her team, successfully brought LYSOGENE's first product SAF-301 into the clinic, in less than five years
 - ✓ Before creating LYSOGENE, Karen's entrepreneurial experience already included successfully founding and running a business consultancy specialized in the financial industry
 - ✓ Prior to that, Karen was a manager at Arthur Andersen where she began her career. At Andersen, she specialized in international M&A related transaction services for major tier one clients
 - ✓ From 2008 to 2009, Karen served as a Member of the Pediatric Committee of the European Medicine Agency as a Patient Representative
 - ✓ In 2008, she also served on the French Ethical Review Board CCPPRB Hôpital Ambroise Paré Boulogne
 - ✓ Karen was also involved with several not-for-profit organizations engaged in advocacy and research in the field of rare diseases such as Alliance SANFILIPPO and EURORDIS, where she served on Board as Treasurer from 2010 to 2011.
 - ✓ Karen received her M.Sc., MBA from ESSEC, France (1993) and a pre-doctoral Research Degree (DEA) in Economics at Université Paris-8 (1994)

GENESIS

- July 2005: Karen & Gad Aiach parents to a child with Sanfilippo Syndrome type A, a lethal disease with no treatment option, decide to make a difference and conquest a cure for that disease
- January 2006: Inception of Alliance Sanfilippo (association Loi 1901)
 - Chairman: Karen AIACH (ESSEC + DEA, Andersen, Armadys Conseil) – Advisor: Olivier DANOS (PhD, Descartes, Harvard, Pasteur, Genethon, UCL, Kadmon)– Treasurer: Gad AIACH (Paris Dauphine, Andersen, Siparex, Deutsche Bank (M&A), Colombus Audit & Expertise)
- December 2006: Decision is made to launch a gene therapy program mainly based on promising PoC
- First Half 2007:
 - Planning & Budget (Karen AIACH, Olivier DANOS & Experts)
 - Financial support: Alliance SANFILIPPO – AFM Téléthon
 - Additional support (Fondation Sanfilippo Suisse)
- Second Half 2007: Effective Start of Alliance SANFILIPPO's Gene Therapy Program

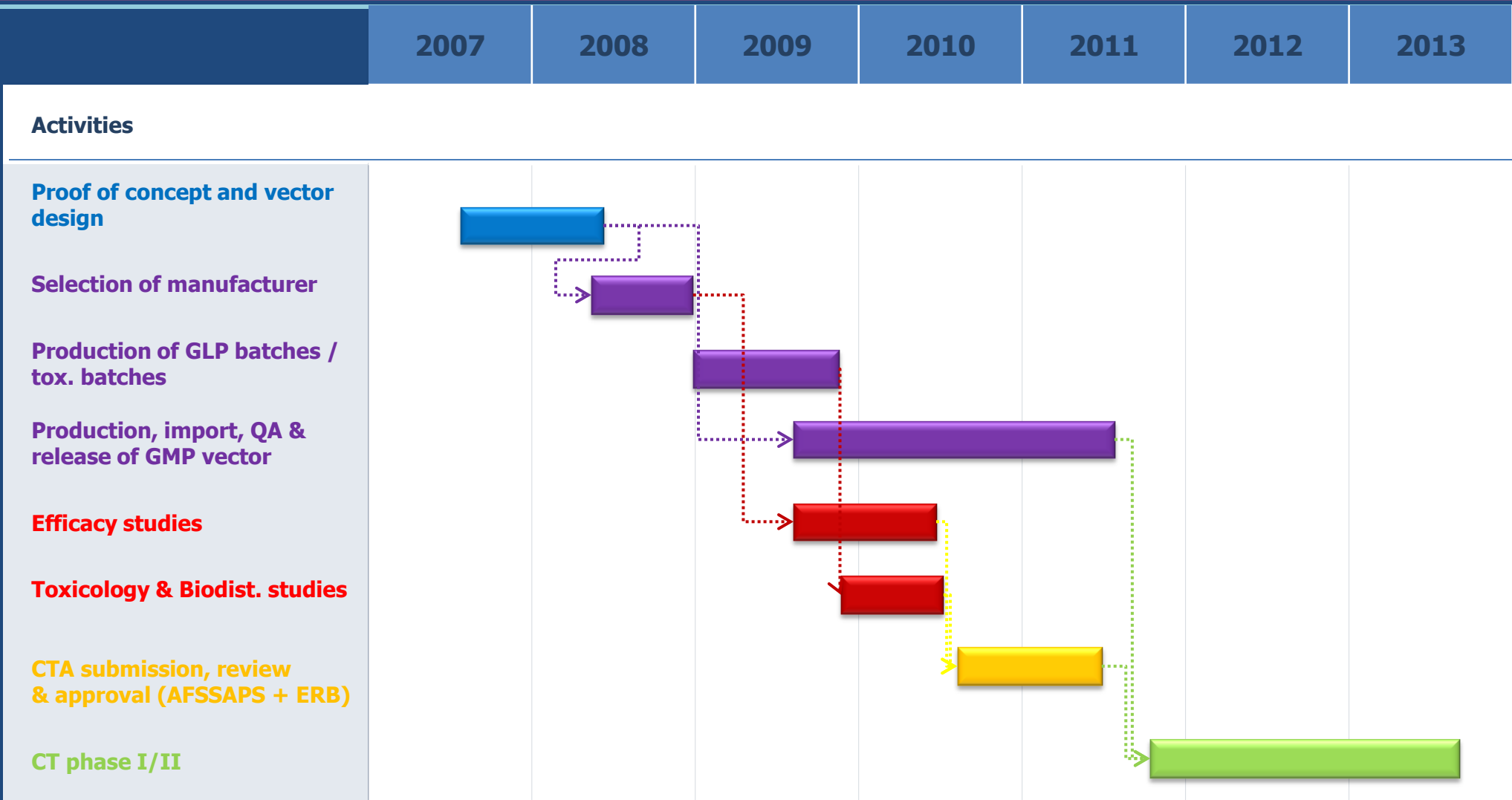
MAIN ACHIEVEMENTS TO DATE

- 2007 – 2009: The intense work lead by Alliance SANFILIPPO and its scientific academic partners: study designs, product manufacturing..., is being extremely productive
 - Decision to move ahead towards clinical trial with an appropriate structure
- Situation in early 2009: no candidate for a transfer into the clinical stage
 - Money
 - Motivation
- May 2009: Inception of a sponsor company, Lysogene (formerly Sanfilippo Therapeutics) , an Alliance Sanfilippo owned subsidiary
 - Mission: finalize the translational program – organize and perform a Phase I/II trial
 - Structuration:
 - Karen Aiach
 - Columbus Audit & Expertise
 - Funding
 - ✓ Founders
 - ✓ Wealthy individuals
 - ✓ Alliance Sanfilippo
 - ✓ AFM – Téléthon
 - ✓ Fondation Sanfilippo Suisse

MAIN ACHIEVEMENTS TO DATE AND VISION

- 2009 – 2010: Preparation of the trial / submission
- 2011 – 2013: Clinical trial completion
- 2013: Expanding the company into a commercially viable one

SAF-301 – TRANSLATION FROM BENCH TO BEDSIDE IN LESS THAN 5 YEARS



PAGs and RESEARCH, UP TO CLINICAL TRIALS AND MA

- Raising awareness
- Initiating
- Funding
- Contributing

- → Catalyzing

OUR VISION

- In 2018, LYSOGENE wants to be one of the worldwide top commercial gene therapy companies in the orphan drug space
- Taking advantage of a Global Gene Therapy Market holding tremendous therapeutic potential
 - With one product on the market, one additional product in clinical development, a potential to expand to additional diseases
- Leveraging on its established and growing platform infrastructure and assets
- Expanding its impact through scientific and societal innovation
- Patients at every stage of the development:
 - Clinical design cf. Lysogene's QASAS Program (Quality of life Assessment in SANfilippo Syndrome)
 - Ethical framework and documentation
 - Evaluation
 - Recrutement
 - Communiacion

THANK YOU FOR YOUR ATTENTION!

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www.lysogene.com