

## INFO GRAPHICS – WORLDWIDE DISTRIBUTION OF NEUROLOGICAL STARTUPS

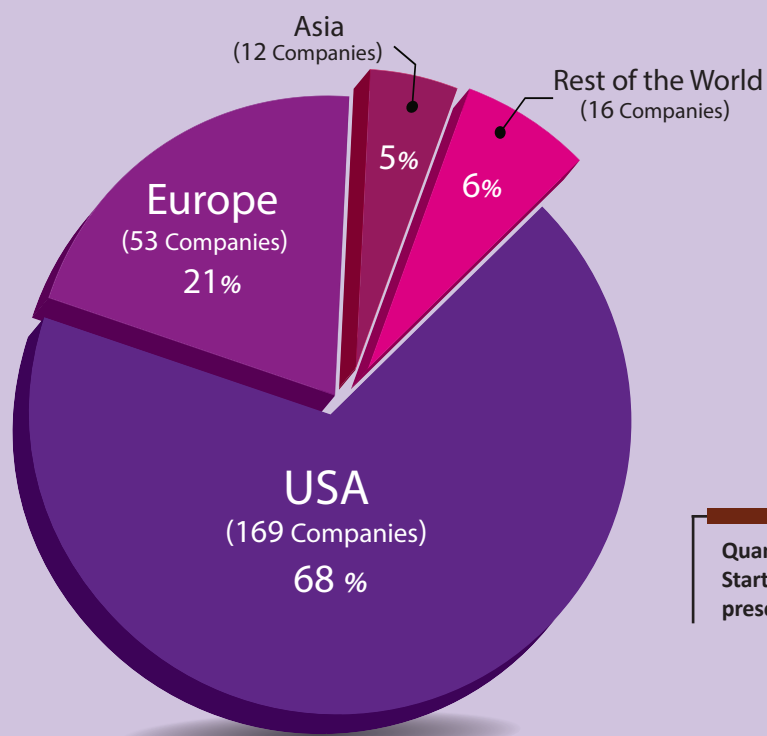


Fig A..1

Quantitative Estimation of Top 250 Active Neurology Startups established between 2010 to mid 2016; present in U.S.A vs. Europe vs. Asia vs. Rest of the World.

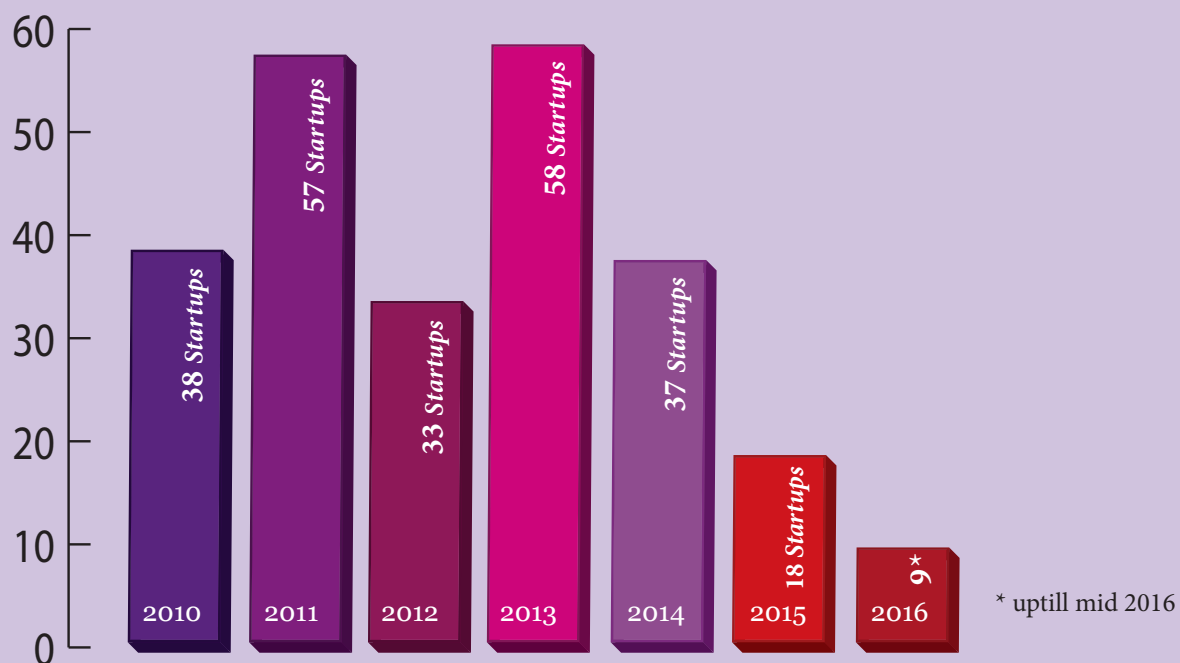
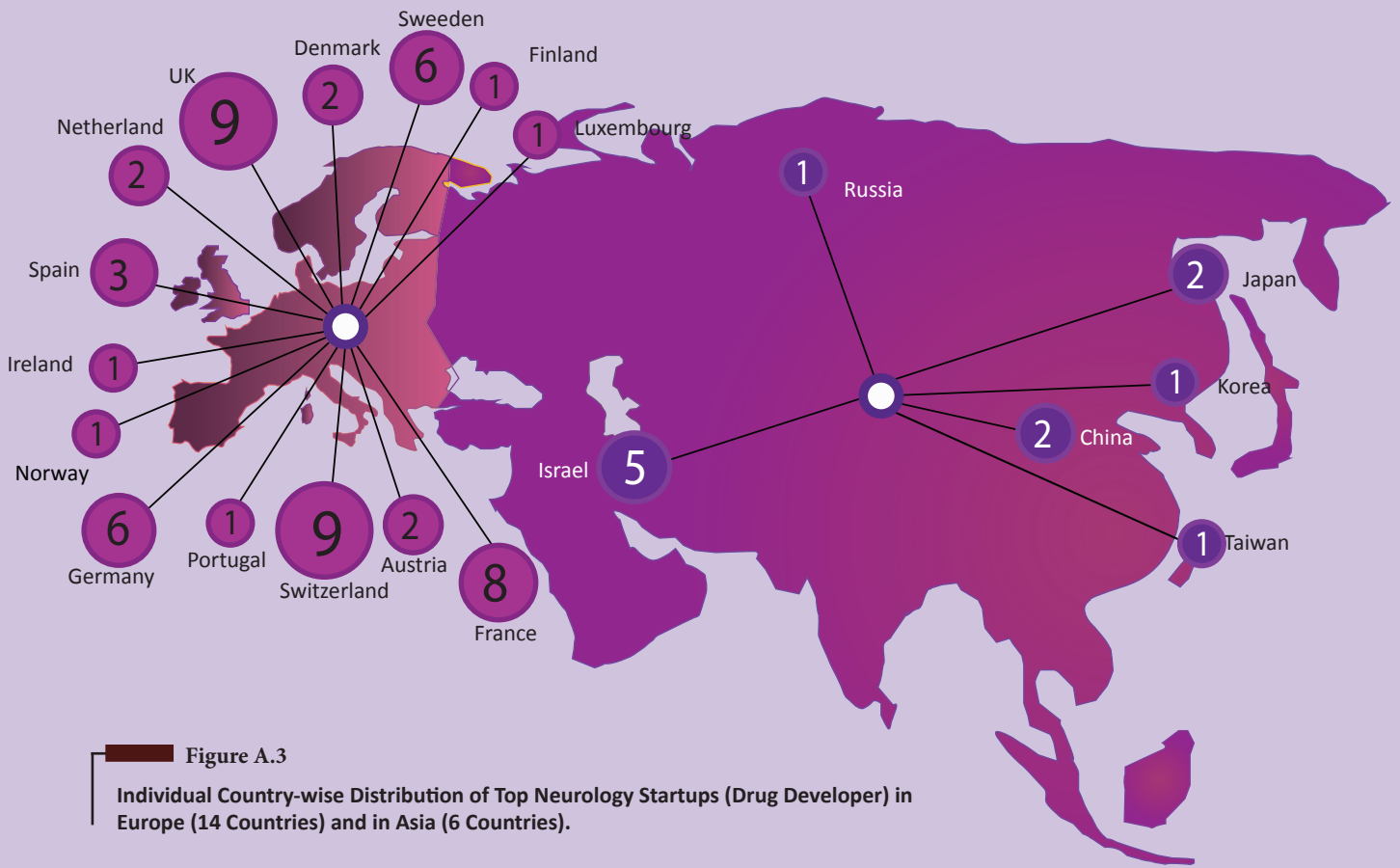


Fig A..2

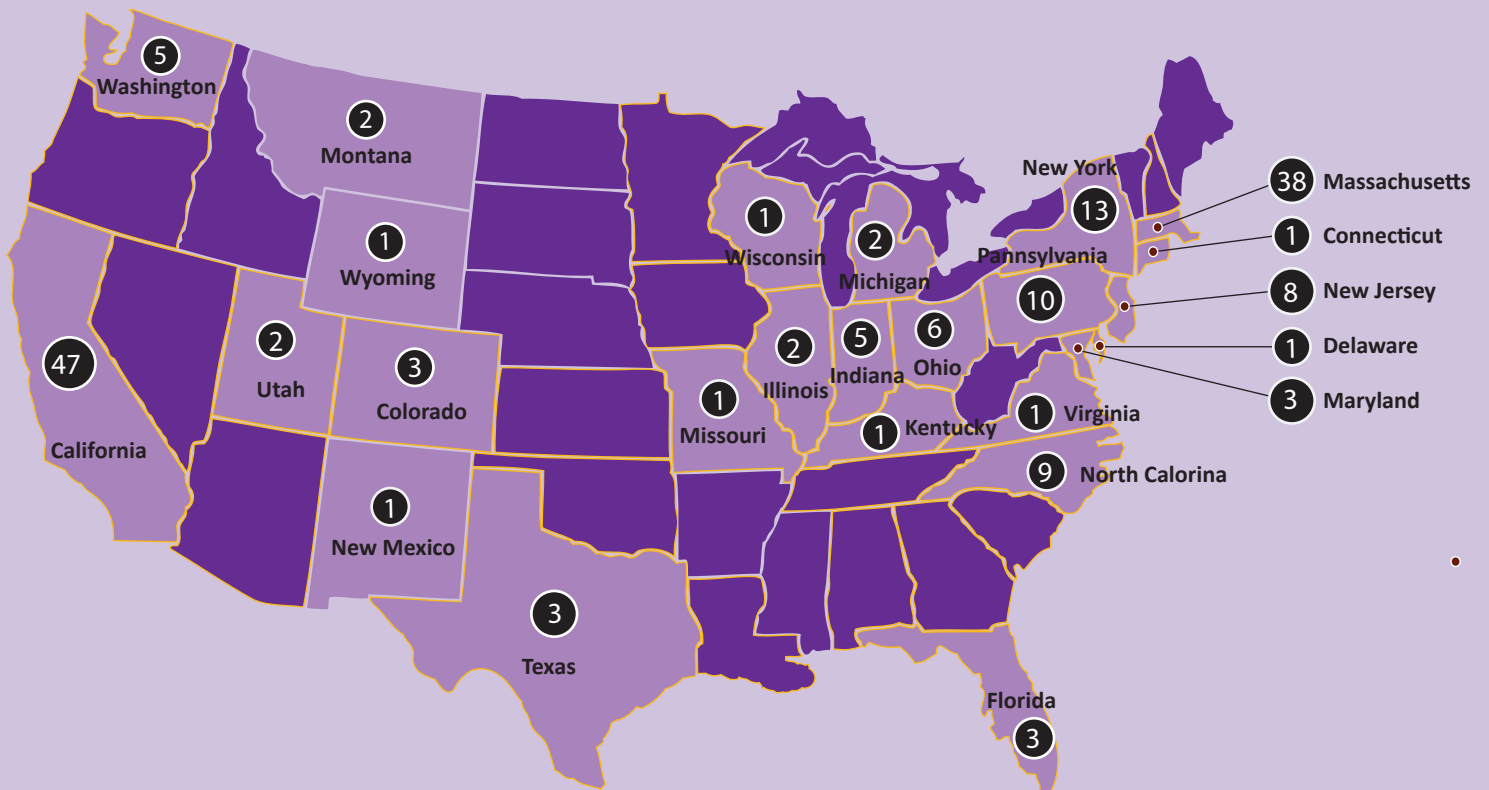
Yearwise Establishment of Top 250 Active Neurology Startups; between 2010 to mid 2016.

**INFOGRAPHICS- COUNTRY WISE DISTRIBUTION OF COMPANIES IN EUROPE & ASIA**



**Figure A.3**

Individual Country-wise Distribution of Top Neurology Startups (Drug Developer) in Europe (14 Countries) and in Asia (6 Countries).



**Figure A.4**

State-wise Distribution of Neurology Startups (established between 2010 - mid 2016) in U.S.A.

## Intelligence - TOP 20 INNOVATIVE STARTUPS CAPABLE OF CHANGING NEUROLOGY DRUG DEVELOPMENT

Table No. A.2

| Sl No | COMPANY NAME (ALPHABETICALLY)               | DESCRIPTION   |
|-------|---|---|
| 1.    |   |   |
| 2.    | <b>Anagenesis Biotechnologies</b><br>France | Anagenesis' proprietary technology enables to regenerate muscle cells (satellite cell) from stem cells, which allows the company to generate unlimited quantities of Paraxial Mesoderm Multipotent Cells (P2MCs) and differentiated muscle cells by in vitro successfully recapitulating each step of the embryonic and foetal myogenesis.  |
| 3.    |   |   |
| 4.    | <b>Celvive Inc.</b><br>U.S.A.               | Celvive has generated a closed system (The StemCell bag™) that consists of sterile, single-use disposable sets of bags and attachments connected to a microprocessor-controlled instrument to enable the isolation of matrix stem cells in a functionally closed system. These autologous stem cells are separated in the StemCell® bag can be injected back into the patient via intrathecal injections. |
| 5.    | <b>Circuit Therapeutics</b><br>U.S.A.       | Circuit's patented Optogenetics technology is a transformational technology that allows control of specific neurons and drives their activity. It requires a light-sensitive protein, or opsin, and light, depending on the wavelength, light can either activate neurons with excitatory opsins, inhibit neurons with inhibitory opsins.   |
| 6.    |   |   |
| 7.    |   |   |
| 8.    |   |   |
| 9.    | <b>Kallyope, Inc.</b><br>U.S.A.             | Kallyope's platform is dedicated to unlocking the therapeutic and nutritional product potential of the gut-brain axis, which offers opportunities to access and influence brain centers involved in fundamental human processes.  |
| 10.   |   |   |
| 11.   |   |   |
| 12.   | <b>Myelin Therapeutics</b><br>U.S.A.        | Myelin Therapeutics' uses AAV-mediated technology to deliver a natural protein to simulate an endogenous mechanisms of brain repair in genetically-inherited pathologies of the central and peripheral nervous systems.   |
| 13.   |   |   |
| 14.   |   |   |
| 15.   |   |   |
| 16.   |   |   |
| 17.   |   |   |
| 18.   |   |   |
| 19.   |   |   |
| 20.   |   |   |

## Intelligence - NEUROLOGY STARTUPS VENTURE FUNDING - 2015

### SERIES A/B/C

Table No. A.6

| Sl No | COMPANY NAME                  | COUNTRY     | SERIES        | FUNDS RAISED   | KEY INVESTORS  |
|-------|-------------------------------|-------------|---------------|----------------|--|
| 1.    |                               |             |               |                |  |
| 2.    | AMO Pharma Ltd.               | U.S.A       | Series A      | \$25 Million   | Woodford Investment Management   |
| 3.    | ASCEnuron SA                  | Switzerland | Series A      | CHF 30 Million | Sofinnova Partners. Other investors includes SR One, Kurma Partners and Johnson & Johnson Innovation and MS Ventures.  |
| 4.    | Autifony Therapeutics Limited | U.K         | Series A Ext. | £8 Million     | SV Life Sciences, Imperial Innovations, Pfizer Venture Investments, the International Biotechnology Trust PLC and UCL Business.  |
| 5.    |                               |             |               |                |  |
| 6.    | Cortexyme, Inc.               | U.S.A       | Series A      | \$15 Million   | Pfizer Inc., Takeda Pharmaceutical Company Ltd., Dolby Family Ventures.  |
| 7.    | Denali Therapeutics, Inc.     | U.S.A       | Series A      | \$127 Million  | Fidelity Biosciences, ARCH Venture Partners, Flagship Ventures and the Alaska Permanent Fund.  |
| 8.    | Iron Horse Therapeutics, Inc. | U.S.A       | Series A      | \$10 Million   | Avalon Ventures and GlaxoSmithKline  |
| 9.    |                               |             |               |                |  |
| 10.   |                               |             |               |                |  |
| 11.   |                               |             |               |                |  |
| 12.   |                               |             |               |                |  |
| 13.   |                               |             |               |                |  |
| 14.   |                               |             |               |                |  |
| 15.   |                               |             |               |                |  |
| 16.   |                               |             |               |                |  |
| 17.   |                               |             |               |                |  |
| 18.   | Tal Medical, Inc.             | U.S.A       | Series A      | \$14 Million   | PureTech Ventures  |
| 19.   |                               |             |               |                |  |
| 20.   |                               |             |               |                |  |
| 21.   | Ovid Therapeutics, Inc.       | U.S.A       | Series B      | \$80 Million   | Fidelity, Cowen Private Investments, Sanofi-Genzyme BioVentures, Tekla Capital, Sphera Global Healthcare Fund, Jennison Associates, Redmile Group, Double-Line Equity Healthcare Fund. |
| 22.   |                               |             |               |                |  |

# Agilis Biotherapeutics, LLC

Year Founded **2013**

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## KEY HIGHLIGHTS

- A** Agilis Biotherapeutics, is a biotech company, focuses on designing and engineering DNA-based therapeutics for patients affected by life-threatening or fatal rare diseases.
- D** In May 2015, The University of South Florida granted Agilis an exclusive, worldwide rights to develop and commercialize UBE3a gene therapies to treat Angelman syndrome.
- D** In Sep 2015, University of South Florida grants Agilis exclusive, worldwide rights to develop and commercialize Reelin (RELN) replacement technologies to treat cognitive disorders.
- D** In Jan 2016, Agilis acquired exclusive, worldwide license for AADC gene therapy from National Taiwan University developed by Dr. Paul Hwu, Prof. of Pediatrics at NTU Children's Hospital with adeno-associated virus (AAV) delivering the human AADC gene.
- F** In Dec 2013, Agilis Biotherapeutics raised \$ 8 Mn. in series A financing round.
- P** In Dec 2013, Agilis and Intrexon Corp. (NYSE:XON), signed an exclusive channel collaboration to develop DNA based therapeutics for Friedreich's ataxia. Under the terms, Agilis' novel DNA based therapeutics will utilize Intrexon's UltraVector® platform and RheoSwitch Therapeutic System® (RTS®) to develop gene therapies and genetically modified cell therapies for treating FRDA.

## NEUROLOGY DRUG DEVELOPMENT PIPELINE

| Name      | Target     | Phase       | Indication          | Molecule Type |
|-----------|------------|-------------|---------------------|---------------|
| AGIL-AADC | DDC gene   | Phase II    | AADC Deficiency     | Gene Therapy  |
| AGIL-FA   | FXN gene   | Preclinical | Friedreich's Ataxia | Gene Therapy  |
| AGIL-AS   | UBE3A gene | Preclinical | Angelman Syndrome   | Gene Therapy  |
| AGIL-RLN  | RELN gene  | Preclinical | Cognitive Disorders | Gene Therapy  |

- ★ AADC Deficiency is a rare CNS disorder arising from reductions in the enzyme aromatic L-amino acid decarboxylase (AADC) that result from mutations in the dopa decarboxylase (DDC) gene.
- ★ Agilis has partnered with T4TOP Clinical Research and National Taiwan University to investigate a gene therapy with adeno-associated virus (AAV) delivering the human AADC gene.
- ★ AGIL-AS, a gene therapy, design for the treatment of Angelman syndrome which is caused by Ubiquitin protein ligase E3A (UBE3A) gene, currently in Preclinical development.
- ★ AGIL-FA is designed for the treatment of Friedreich's ataxia, a rare genetic neurodegenerative disease, caused by a single genetic defect in the FRX gene.
- ★ These FRX gene leads to reduced production of frataxin, a mitochondrial protein that is important for iron metabolism, which results in a physically debilitating, life-shortening condition.
- ★ AGIL2AS is being developed for the treatment of Angelman syndrome (AS), a neuro genetic disorder characterized by severe intellectual and developmental disability. AGIL2AS has received an orphan drug status from the US FDA and EU for the treatment of Angelman syndrome.

## CORPORATE PROFILE

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