Bliographical Sketch

Position/Title: Director of Research CNR, Institute of Neuroscience and Head of the "Stem Cells and Neurogenesis" Unit, San Raffaele Scientific Institute, Milan
Education/Training: BSc, 1993, University of Bologna
PhD, 1997, Genetic Sciences, San Raffaele Scientific Institute, Milan

Broccoli Vania is a neuroscientist of the CNR Institute of Neuroscience and is leading the Research Unit of "Stem Cells and Neurogenesis" at the San Raffaele Scientific Institute in Milan (Italy). He has unraveled some of the molecular mechanisms that control key processes of brain development including neural stem cell identity, neural commitment and migration, neural network establishment and function. Lately, his group has developed new technologies of direct cell reprogramming and generated human neurons suitable for convenient *in vitro* model of Parkinson's disease and other neurological disorders. His lab is also actively working on novel gene therapy strategies for brain diseases by using novel engineered synthetic AAVs and CRISPR/Cas9 gene modification approaches.

Positions and Honors.

2006 - 2010: **Group Leader**, San Raffaele Scientific Institute, Milan, Italy.
2010 - present: **Head of Research Unit**, San Raffaele Scientific Institute, Milan, Italy.
2015 - present: **Head of Research Unit**, CNR Institute of Neuroscience, Milan, Italy.
2014 - present: **Associated scientist**, Sant'Anna School of Advanced Studies, Life Science Center, Pisa, Italy.

- Member of the Armenise Foundation's Italian Scholarship Advisory Committee – Armenise Harvard Foundation (2015 - 2018)
- Member of the evaluating committee for the Consolidator European Research Council (ERC) awards, Panel LS7 (2015 - present).
- Member of the Reviewer Scientific Panel of the California Institute of Regenerative Medicine (CIRM) (2013 – present), California, USA
- Member of the European Scientific Advisory Board of the FP7-ERANET Neuron-II program (2011 - 2018)
- Member of the external Scientific Advisory Board of the University of Lund (Sweden) (2012 – 2016) – Life Science
- Recipient of the European Research Council (ERC) Advanced grant (2014 – 2019).
- Supervisor of 11 PhD students who have finished their thesis.
- Editor for the "Le Scienze" monothematic issue on "Brain diseases": Le Scienze N.119 (2006)
- 1999 - HFSP long-term fellowship
- 1998 - EMBO short-term fellowship

B. 10 selected peer-reviewed publications 2015-2018

- **Broccoli V**, Luoni M. (2018). Recipes for Making Neurons using Combinatorial Forward Genetics. Cell Stem Cell 23(1):13-14.

- Iannielli A, Bido S, Folladori L, Segnali A, Cancellieri C, Maresca A, Massimino L, Rubio A, Morabito G, Caporali L, Tagliavini F, Musumeci O, Gregato G, Bezard E, Carelli V, Tiranti V, **Broccoli V**. (2018).*Pharmacological Inhibition of Necroptosis Protects from Dopaminergic Neuronal Cell Death in Parkinson's Disease Models.* Cell Reports 22(8):2066-2079.

- Giannelli SG, Luoni M, Castoldi V, Massimino L, Cabassi T, Angeloni D, Demontis GC, Leocani L, Andreazzoli M, **Broccoli V**. (2018). *CRISPR/Cas9 selective and effective targeting of the P23H Rhodopsin disease mutant allele for the treatment of Retinitis Pigmentosa.* Human Molecular Genetics 27(5):761-779.

- Morabito G, Giannelli S, Ordazzo G, Bido S, Castoldi V, Indrigo M, Cabassi T, Cattaneo S, Luoni M, Cancellieri C, Sessa A, Bacigaluppi M, Taverna S, Leocani L, Lanciego JL, **Broccoli V.** (2017). *Global-scale control of gene expression in the adult mouse nervous system by a single AAV- PHP.B systemic injection enables GBA1 gene therapy for complete protection from synucleopathy.* Molecular Therapy 25(12):2727-2742.

- Mazzara PG, Massimino L, Pellegatta M, Ronchi G, Ricca A, Iannielli A, Giannelli SG, Cursi M, Cancellieri C, Sessa A, Del Carro U, Quattrini A, Geuna S, Gritti A, Taveggia C, **Broccoli V**. (2017). *Two factor-based reprogramming of rodent and human fibroblasts into Schwann cells.* Nature Communications 8:14088.

- Rubio A, Luoni M, Giannelli SG, Radice I, Iannielli A, Cancellieri C, Di Berardino C, Regalia G, Lazzari G, Menegon A, Taverna S, **Broccoli V.** (2016). *Rapid and efficient CRISPR/Cas9 gene inactivation in human neurons during human pluripotent stem cell differentiation and direct reprogramming*. Scientific Reports 6:37540.

- Vasconcelos FF, Sessa A, Laranjeira C, Raposo AA, Teixeira V, Hagey DW, Tomaz DM, Muhr J, **Broccoli V,** Castro DS. (2016). *MyT1 Counteracts the Neural Progenitor Program to Promote Vertebrate Neurogenesis.* Cell Reports 17(2):469-483.

- Orellana D, Santambrogio P, Rubio A, Di Meo I, Tiranti V*, **Broccoli V***, Levi S*. *Co-corresponding authors. *CoA rescues mitochondrial and metabolic deficits in PANK2 mutant mice and human iPSC-derived neurons.* EMBO Molecular Medicine 8(10):1197-1211.

- Gautier CA, Erpapazoglou Z, Mouton-Liger F, Muriel MP, Cormier F, Bigou S, Duffaure S, Girard M, Foret B, Iannielli A, **Broccoli V**, Dalle C, Bohl D, Michel PP, Corvol JC, Brice A, Corti O. (2016). *The endoplasmic reticulum-mitochondria interface is perturbed in PARK2 knockout mice and patients with PARK2 mutations.* Human Molecular Genetics 25(14):2972-2984.

- Colasante G, Lignani G, Rubio A, Medrihan L, Yekhlef L, Sessa A, Massimino L, Giannelli SG, Sacchetti S, Caiazzo M, Leo D, Alexopoulou D, Dell'Anno MT, Ciabatti E, Orlando M, Studer M, Dahl A, Gainetdinov RR, Taverna S, Benfenati F, **Broccoli V**. (2015). *Rapid Conversion of Fibroblasts into Functional Forebrain GABAergic Interneurons by Direct Genetic Reprogramming.* Cell Stem Cell 17(6), 719-734.