

033 Treating Neuroblastoma via BMP4

- ✓ N-myc Amplified Neuroblastoma Cells as therapeutic target
- ✓ BMP4a for Specific Inhibition of Histone Deacetylases (HDAC)
- ✓ Effectiveness at very low Level (nanomolar)

The Technology

Bone morphogenetic protein 4 (BMP4) was identified with the capability to induce growth arrest, differentiation, and apoptosis that assume a potential therapeutic benefit for treatment of neuroblastomas, because healthy normal cells are not affected by BMP4. This opens a therapeutic approach with obviously less side effects compared with current standard therapies.

Background

Neuroblastoma is the most common extracranial solid cancer in childhood and the most common cancer in infancy, with an annual incidence of about six hundred and fifty cases per year in the U.S. and a hundred cases per year in the U.K. Nearly half of neuroblastoma cases occur in children younger than two years. Between 20% and 50% of high-risk cases do not respond adequately to induction high-dose chemotherapy and are progressive or refractory. Relapse after completion of frontline therapy is also common.

The majority of survivors have long-term effects from the treatment. An estimated two of three survivors of childhood cancer will ultimately develop at least one chronic and sometimes life-threatening health problem.

Advantages

Specific inhibition of histone deacetylases by BMP4 efficiently restores aberrant signaling pathways and biological features in experimental neuroblastoma at nanomolar concentration without unfavorably targeting untransformed cells. In addition, recombinant manufactured BMP4 is already established for other indications (osteoporosis and type 2 diabetes), which should support the approval for this new indication.

Commercial Opportunity

- ✓ Development of BMP4 as a new therapeutic drug candidate against later stage of neuroblastoma.

Intellectual Property

An international patent application was filed (WO 2009/101012). The Europe equivalent EP 2259792 has been granted October 5, 2011 and validated for CH, GB, DE, FR, see

http://worldwide.espacenet.com/searchResults?NUM=EP2259792&DB=EPODOC&locale=en_EP&ST=number&compact=false. The USA equivalent US S-

2011-0020334-A1 is pending, see

http://worldwide.espacenet.com/searchResults?NUM=US20110020334&DB=EPODOC&locale=en_EP&ST=number&compact=false

Contact:

technology transfer heidelberg GmbH

Im Neuenheimer Feld 672

D-69120 Heidelberg

Germany

Email: tt-team@med.uni-heidelberg.de



UniversitätsKlinikum Heidelberg

**Bone morphogenetic protein 4 (*BMP4*) –
the strongest and among the earliest up-regulated genes**

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