

## Pediatric Cancer

### Expanded Access Guidelines

April 2017

#### Background

The FDA issued updated guidelines to “expanded access” (often known as compassionate use) for patients to gain access to treatment with an investigational product when they have not responded to all currently available approved products and a clinical trial is not available or they do not meet the criteria for any clinical trial. See FDA guidelines 21 CFR part 312 subpart 1.

For a single patient to be treated by a licensed physician the physician must apply for an IND using form FDA 3926, needs to obtain IRB approval from their facility and needs a letter of authorization (LOA) from the company supplying the investigational product.

#### Important Information

1. **What is BIO-11006?** It is an investigational peptide medication delivered by nebulizer which has shown activity in the laboratory and in animal studies in certain types of cancer. It has been granted FDA acceptance to begin studies in adult lung cancer. BIO-11006 has been given to over 200 patients with COPD (chronic bronchitis and emphysema) and been shown to have a good safety profile at the recommended dose. It has not been given to children or cancer patients in controlled clinical studies.
2. **What types of childhood cancers has it been tested on in the laboratory?** It has shown an effect on isolated rhabdomyosarcoma, neuroblastoma, sarcoma and glioma cells (in vitro).
3. **My child has cancer and has not responded to available treatment – are they a candidate for compassionate use?** Your treating clinical oncologist should determine if the child is a candidate using the above guideline from the FDA.
4. **How do we contact you for information ?** Please ask your physician to email to [CompassionateUse@livelikebella.org](mailto:CompassionateUse@livelikebella.org)
5. **Are there costs for the patient if the drug is provided?** The company has committed to provide the drug free of charge. However supplies are limited.
6. **Will the drug help extend or save my child’s life?** We do not know the effectiveness of the drug in a child with cancer.