

Brilacidin (PMX-30063) Antibiotic Fact Sheet

www.polymedix.com

February 2013

New Class of Antibiotic: *Defensin-Mimetic*

Infections are a leading cause of death. *70% of infections in the U.S. are resistant to at least one antibiotic drug—creating a serious global medical problem.* MRSA infections alone have increased 700% over 4 years. Annually in the U.S., 14.2 million people are treated for skin and skin structure infections and 7 million for MRSA infections, costing the U.S. healthcare system approximately \$17 billion in direct costs.

PolyMedix has developed a completely novel class of antibiotic drugs, defensin-mimetics: *small-molecules that imitate natural human immunity.* With a completely different mechanism of action from other antibiotics, these unique compounds function in a way which makes *bacterial resistance unlikely to develop.* The first compound in this series which has recently completed a **Phase 2 clinical trial** for the treatment of *Staphylococcus* infections, including MRSA, is **brilacidin** (also known as PMX-30063). In the trial, brilacidin demonstrated consistently high clinical response rates comparable to those of daptomycin (the active control), and was shown to be safe and generally well-tolerated.

Phase 2 Clinical Trial

PolyMedix enrolled 215 patients in a multinational Phase 2 clinical trial to evaluate the safety and efficacy of brilacidin in treating patients that have acute bacterial skin and skin structure infections (ABSSSI) caused by either MSSA or MRSA.

Patients were randomized into four different arms. Three arms were administered low, medium or high doses of brilacidin (0.4 mg/kg on day one followed by 0.30 mg/kg daily for four days; 0.75 mg/kg on day one followed by 0.35 mg/kg daily for four days; or 1.0 mg/kg on day one followed by 0.35 mg/kg daily for four days) plus two days of placebo for a total of seven days. The fourth arm was administered daptomycin daily for seven days (per label recommendations).

The trial followed the most recent guidance issued by the Food and Drug Administration (FDA) and assessed patients at Days 3, 7, 10 and 28.

The trial achieved its objectives of meeting efficacy and safety in all evaluated doses of brilacidin. All regimens of brilacidin for all patient populations and time points showed early, high and sustained clinical responses. These clinical response rates observed in the study suggest the potential for exploration of shorter treatment regimens. In the study, brilacidin appeared to be safe and was generally well-tolerated.

Efficacy

As show below, per the study analysis plan, patients in the iTT Population (all-comers into the study) showed early, high and sustained clinical responses.

Clinical Response	Low dose n=52	Medium dose n=54	High dose n=54	Daptomycin n=55
Day 3	94.4%	90.7%	81.5%	90.6%
Day 7	87.0%	92.6%	83.3%	96.2%
Sustained Response	Low dose n=52	Medium dose n=54	High dose n=54	Daptomycin n=55
Day 10	90.2%	91.8%	95.5%	97.9%
Day 28	95.7%	89.6%	95.6%	98.0%

Safety

The most common adverse event experienced by patients in the study was numbness and tingling, felt by 65%-87% of treated patients. The majority of the cases were rated as mild and no patient discontinued due to just numbness and tingling. Excluding the numbness and tingling, the treatment-related adverse events were 9.6%, 5.6%, and 7.4% for the low, medium and high dose of brilacidin vs. 10.9% for daptomycin. Eight patients discontinued treatment due to treatment-related adverse events. Three patients that received brilacidin experienced a treatment-related serious adverse event Two of these patients discontinued treatment due to hypertension (one medium dose and one high dose).

Next steps

Based on the successful results of this trial, which suggests that shorter courses of therapy and lower doses could be effective, a dose-optimization Phase 2B study is planned. The Phase 2B will study lower doses and shorter courses of therapy.

Brilacidin (PMX-30063) Antibiotic Fact Sheet

www.polymedix.com

February 2013

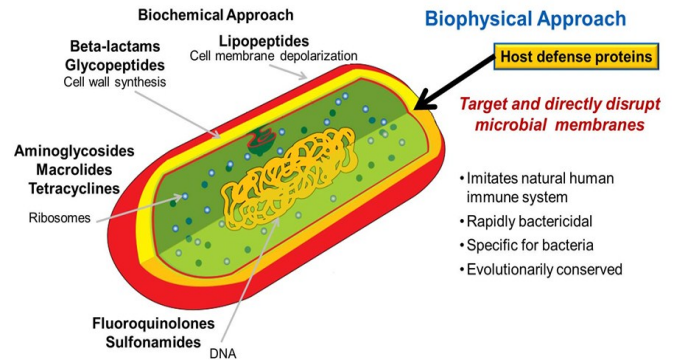
Defensin-Mimetic Attributes

- A unique mechanism of action fundamentally different from all current systemic antibiotics – direct disruption of bacterial cell membranes, the same as our own natural defensins - which makes *bacterial resistance unlikely to develop*
- *Selectively targets bacteria* and not mammalian cells
- Potent, broad spectrum activity against multiple Gram-positive and Gram-negative bacteria
- Bactericidal, not simply bacteriostatic like other antibiotics
- Short course of therapy - Phase 2 trial demonstrated effectiveness with 5 days (vs. 7 for daptomycin), and PK/PD modeling and animal studies predict single dose efficacy, which will be studied in the next clinical trial.
- Many additional indications and applications are possible, such as bacteremia and pneumonia with the i.v. formulation, and oral mucositis in cancer patients with a topical oral rinse.

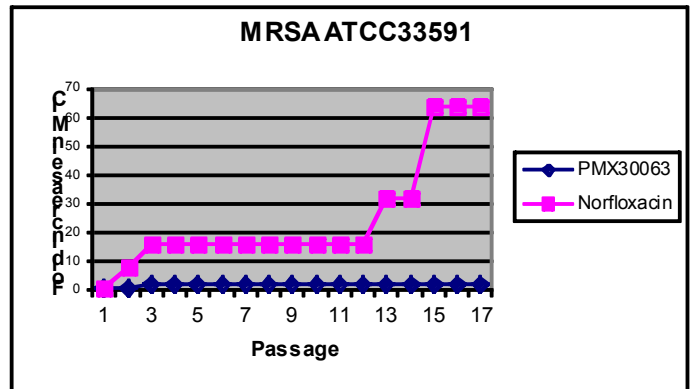
Active against multiple Gram-positive and Gram-negative bacteria

Our defensin mimetics provide potent, *broad spectrum activity* against multiple Gram-positive bacteria including MRSA, *Enterococcus faecium*, and Gram-negative bacteria including *Escherichia coli*, and *Klebsiella pneumonia* (NDM-1); all of which have become resistant to many antibiotic drugs.

Unique Mechanism of Action Fundamentally Different from ALL Current Systemic Antibiotics



Bacterial Resistance Unlikely

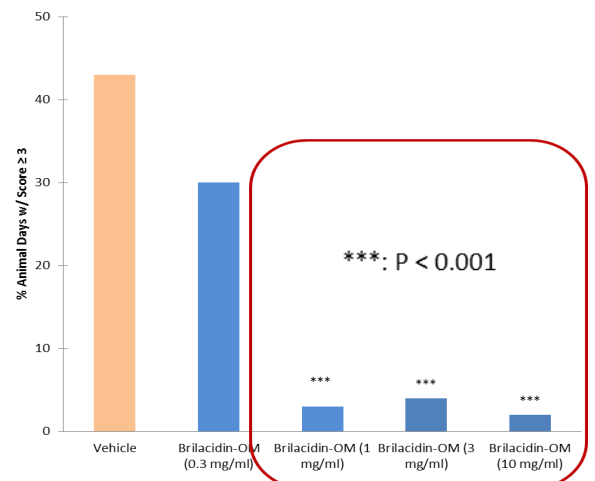


Serial passage resistance studies are used to demonstrate the potential for bacterial resistance to develop to antibiotics. The graph shows brilacidin compared to a conventional antibiotic, norfloxacin for the development of resistance against MRSA. **With brilacidin, no bacterial resistance was seen in up to 40 serial passages.**

Brilacidin for Oral Mucositis

PolyMedix is developing brilacidin as an oral rinse for the prevention of oral mucositis in cancer patients. Oral mucositis is a common, potentially severe and debilitating inflammation and ulceration that can occur in the mouth as a side effect of some chemotherapy and radiation therapy treatments for cancer. It afflicts approximately 450,000 cancer patients each year in the United States, and can affect the course and outcome of cancer therapy.

In two animal models where oral mucositis was induced by acute and fractionated radiation, brilacidin was administered as an oral topical rinse. The results showed that brilacidin statistically significantly reduced the severity and duration of the mucositis in these studies by 90%-95%.



For more information contact: Edward F. Smith - President & CEO/CFO - 484-598-2332
PolyMedix, Inc., 170 Radnor-Chester Rd., Suite 300, Radnor, PA 19087

PolyMedix®