



# NOVEL MEDICAL THERAPY FOR REFRACTORY ENDOMETRIOSIS ASSOCIATED CHRONIC PELVIC PAIN: AN OPEN LABEL TRIAL OF THALIDOMIDE



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## BACKGROUND

Endometriosis affects 2.5-3.3% of reproductive age women and is a common diagnosis among women with chronic pelvic pain. Treatment for endometriosis ranges from conservative medical therapies to radical surgery. Endometriosis is an indication for 25-35% of laparoscopies and 10-15% of hysterectomies each year. Although the majority of women with endometriosis respond to conservative medical therapy, those with persistent pain often undergo hysterectomy. Based on clinical and experimental data that indicate that thalidomide may be a highly effective immune modulator, we sought to investigate the efficacy and tolerability of this novel therapy for endometriosis associated pain patients who desired fertility-sparing treatment after exhausting all other conservative modalities.

## OBJECTIVE

To investigate the efficacy and tolerability of thalidomide as a treatment for endometriosis associated pain patients

## METHODS

Six women with biopsy proven endometriosis and chronic pelvic pain were enrolled from August 2006 to May 2007. Participants had tried conventional therapeutics such as oral contraceptive therapy, GnRh-agonist, and/or treatment with Danazol, but these therapies had failed to treat their symptoms. Thus, they had been offered hysterectomy as a last resort. Enrolled subjects reported daily maximal pain of  $\geq 60$  on the Visual Analog Scale (VAS).

Two of the six eligible women completed the 14 week open label trial with thalidomide (dose range of 50mg to 250mg). Participants were assessed weekly for pain and drug tolerability.

## RESULTS

- Four women dropped out of the study due to domestic matters (n=2) and drug-related side effects (Parkinson type symptoms and gallbladder pain) (n=2).
- The two women who completed the study had large drops in their maximum daily pain (VAS).
- One woman (FA) changed her pain report from baseline of 85 to 0 on the VAS after the 14-week trial.
- The other subject (SM) VAS pain report dropped to 0 (from 100) two-weeks after completing the study.
- Significant drops in percent awake hours in pain were also observed in both subjects; FA dropped from 30% to 5% while SM dropped from 100% to 0%.
- Subject FA had unremitting pain for 5 years. Ten weeks into the trial at 200mg of thalidomide she had a large drop in her VAS pain report and remained pain free for two months after trial termination.
- SM reported unremitting pain for 8 years. While she remained highly symptomatic during the trial (VAS= 100), she had 2 months of pain remission 3 weeks after completing the study.
- The two women who dropped from the study due to side effects at eight weeks indicated improvement in their pelvic pain.
- Two of the participants opted to continue thalidomide therapy off protocol.

**Table 1: Summary of Results**

	Subject 1 (FA)	Subject 2 (SM)
<b>Time of Previous Unremitting Pain</b>	5 years	8 years
<b>Baseline Pain Report (VAS)</b>	85	100
<b>Time when Pain Report (VAS) equaled 0</b>	14 weeks (end of study)	2 weeks post-study
<b>Change in % Wake Hours in Pain</b>	30% $\rightarrow$ 5%	100% $\rightarrow$ 0%
<b>Continued Thalidomide post-study</b>	Yes	Yes

## CONCLUSION

Thalidomide may be useful in treating persistent endometriosis associated pelvic pain. Additional studies are warranted.