# Pegylated liposomal doxorubicin in symptomatic desmoid tumor

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## Background

- Pegylated liposomal doxorubicin (PLD) is one of the available options for patients with desmoid tumor (DT) when active treatment is indicated.
- The aim of this study was to report the results with PLD in a population-based cohort of DT and to investigate the association between radiological response using RECIST, tumor volume measurements and clinical outcome.

## Methods

- Patients with DT treated with PLD at Oslo University Hospital were included.
- PLD 40 mg/m$^2$ was given every 4 weeks for up to 9 cycles.
- Radiological response was determined using RECIST v1.1 and using tumor volume measurements.

## Results

- From 2015 to 2023, 29 patients received PLD. The indication for active therapy was symptoms in 26 patients and symptoms and tumor growth at a critical anatomical site in 3 patients. Active surveillance was the initial strategy in 22 patients.
- The baseline characteristics are summarized in Table 1.
- The median time from diagnosis to active treatment was 6 (range 2-171) months. PLD was given as first line treatment in 26 patients.
- The median number of cycles was 6 (range 3-9) and 24 patients received at least 6 cycles.
- The overall response rate according to RECIST v1.1 was 66% (1 CR and 18 PR; Figure 1). The median time to the best response was 17 (range 3-44) months.
- A reduction in tumor volume was observed in 27 of 29 patients (Figure 2). The median decrease was 78% (range -99% to +114%).
- A high degree of correlation between percent change by RECIST and tumor volume was observed (r=0.856; 95% CI 0.714-0.931; Figure 3).
- Symptom improvement was reported by 19 patients, of whom all had a decrease in tumor size by RECIST (1 CR, 15 PR and 3 SD).
- After a median follow-up of 25 months, PD was recorded in 3 of the 27 patients with initial CR/PR/SD.
- No cardiac toxicity, secondary cancer, or death was registered.

**Table 1. Baseline patient characteristics**

| | |
|---|---|
| Age, years (range) | 36 (18-69) |
| Sex | |
| Female | 19 (66) |
| Male | 10 (34) |
| Follow-up, months (range) | 25 (2-86) |
| Tumor location | |
| Intraabdominal | 8 (28) |
| Abdominal wall | 8 (28) |
| Extremity | 5 (17) |
| Neck | 3 (10) |
| Trunk wall | 3 (10) |
| Intrathoracic | 2 (7) |
| Tumor size, cm (range) | 11.6 (5.0-33.2) |
| Tumor volume, ml (range) | 412 (32-8029) |
| FAP-associated | |
| No | 21 (72) |
| Yes | 8 (28) |
| Multifocal | |
| No | 24 (83) |
| Yes | 5 (17) |

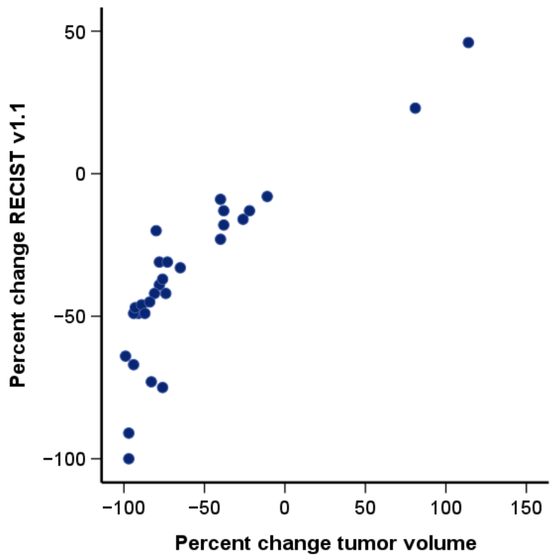


Figure 3. Scatter plot showing the correlation between percent change using RECIST v1.1 and tumor volume.

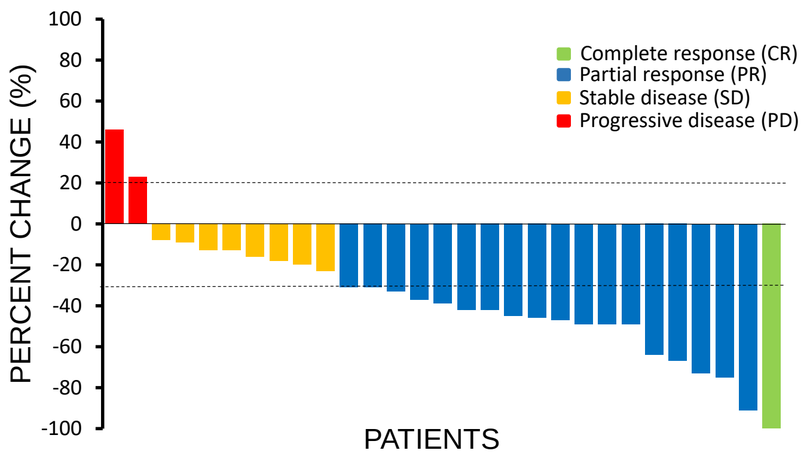


Figure 1. Waterfall plot showing best response according to RECIST v1.1.

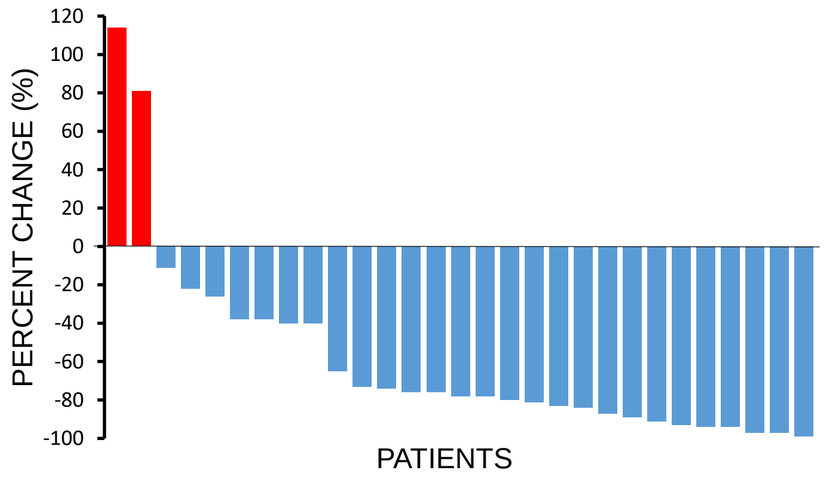


Figure 2. Waterfall plot showing best response according to tumor volume.

## Conclusions

- Treatment with PLD resulted in radiological response and improvement of symptoms in the majority of patients.
- PLD is an effective treatment option in symptomatic DF.
- All patients with symptom improvement had a reduction in tumor size.
- A strong correlation between RECIST and tumor volume measurement was observed.

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