A stochastic search approach to identify subgroups with treatment benefit or harm

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Abstract: Existing statistical methods to identify sub-groups with differential treatment benefit/harm are either based on some parametric structure of the underlying data generation mechanism and/or are estimated through local optimization. We developed a nonparametric approach to identify subgroups through global optimization. Our approach is composed of two steps. In the first step, a discretization procedure creates a number of small sub-populations called "cells" with sufficient granularity, which serves as the building blocks of subgroup identification. In the second step, a simulated annealing algorithm is used to search for combinations of the cells that yield up to three groups: those deriving benefit from the treatment, those harmed by the treatment and the rest. Simulation studies are performed to evaluate the performance of this algorithm as compared with existing methods. A real data example is also presented.