|  |
| --- |
| **Table 1** **Patient characteristic** |
| Characteristic | N | % |
| **Gender** |  |  |
|  　Female | 112 | 67 |
|  　Male | 56 | 33 |
| **Primary / Recurrent** |  |  |
| Primary | 140 | 83 |
| Recurrent | 28 | 17 |
| **Mean age, years (range)** | 39.1 (1 - 88) |
| **Mean size, mm (range)** | 74.7 (13.2 – 255.8) |
| **Site** |  |  |
| 　 Extremities | 50 | 30 |
|  　Abdominal wall | 45 | 27 |
|  　Other trunk | 47 | 28 |
|  Retroperitoneal | 6 | 4 |
|  　Neck | 20 | 12 |
| ***CTNNB1* mutation (n= 154)** |
|  T41A | 91 | 59 |
|  T41I | 5 | 3 |
|  S45F | 17 | 11 |
|  S45P | 6 | 4 |
| H36P | 1 | 1 |
|  Wild type | 34 | 22 |
| **Drug administration** |
|  COX-2 inhibitor | 116 | 69 |
|  Tranilast | 26 | 15 |
|  Tamoxifen | 3 | 2 |
|  None | 42 | 25 |
| **RECIST (n = 156)** |
| CR | 7 | 4 |
| PR | 17 | 11 |
| SD | 64 | 41 |
| PD | 68 | 44 |

CR; complete remission, PR; partial remission, SD; stable disease, PD; progressive disease

|  |
| --- |
| **Table 2 Univariate analysis of factors associated with tumor progression** |
| Factor |  | Non-PD | PD | p value |
| **Gender** | Female | 55 | 50 | 0.14 |
|  | Male | 33 | 18 |  |
| **Age** | < 33 | 31 | 32 | 0.14 |
|  | > 33 | 57 | 36 |  |
| **Size** | < 64mm | 39 | 38 | 0.15 |
|  | > 64mm | 49 | 30 |  |
| **Primary / Recurrent** | Primary | 75 | 55 | 0.47 |
|  | Recurrent | 13 | 13 |  |
| **Site (Neck/Other)** | Neck | 8 | 9 | 0.41 |
|  | Other | 80 | 59 |  |
| **Site (Extremity/Other)** | Extremity | 22 | 25 | 0.11 |
|  | Other | 66 | 43 |  |
| ***CTNNB1* mutation** | S45F | 6 | 9 | 0.19 |
|  **(n = 143)** | Other | 74 | 54 |  |
| **Drug administration** | Presence | 63 | 54 | 0.26 |
|  | Absence | 25 | 14 |  |
| **Conversion to AT** | Presence | 20 | 47 | < 0.0001 |
|  | Absence | 68 | 21 |  |

AT; active treatment

|  |  |  |
| --- | --- | --- |
|  | **Table 3 Univariate and multivariate analysis of factors associated with event-free survival** |  |
| Factor |  | n | Events | Univariate HR(95% CI) | Univariate p value | Multivariate HR(95% CI) | Multivariate p value |
| **Gender** |  |  |  |  |  |  |  |
|  | Female | 113 | 53 | 1.3 (0.8 – 2.2) | 0.29 | NA |  |
|  | Male | 55 | 21 | Ref |  |  |
| **Age** |  |  |  |  |  |  |  |
|  | < 33 | 93 | 39 | 1.3 (0.8– 2.0) | 0.27 | 1.13 (0.69 – 1.83) | 0.63 |
|  | > 33 | 75 | 34 | Ref |  | Ref |  |
| **Size** |  |  |  |  |  |  |  |
| **(n = 162)** | < 64mm | 79 | 32 | Ref | 0.50 | Ref | 0.26 |
|  | > 64mm | 83 | 39 | 1.2 (0.8 -2.0) |  | 1.29 (0.81 – 2.16) |  |
| **Primary / Recurrent** |  |  |  |  |  |  |  |
|  | Primary | 141 | 60 | Ref | 0.93 | NA |  |
|  | Recurrent | 27 | 14 | 0.98 (0.6 – 1.8) |  |  |  |
| **Site (Neck/Other)** |  |  |  |  |  |  |  |
|  | Neck | 20 | 12 | 1.9 (1.0 – 3.5) | 0.042 | 1.80 (0.86 – 3.75) | 0.12 |
|  | Other | 148 | 62 | Ref |  | Ref |  |
| **Site (Extremity/Other)** |  |  |  |  |  |  |  |
|  | Extremity | 50 | 21 | 0.80 (0.5 – 1.3) | 0.38 | NA |  |
|  | Other | 118 | 53 | Ref |  |  |  |
| ***CTNNB1* mutation** |  |  |  |  |  |  |  |
|  **(n = 154)** | S45F | 17 | 13 | 2.4 (1.3 – 4.5) | 0.003 | 1.96 (1.01 – 3.80) | 0.048 |
|  | Other | 137 | 59 | Ref |  | Ref |  |
| **Drug administration** |  |  |  |  |  |  |  |
|  | Presence | 124 | 54 | 0.8 (0.5 – 1.3) | 0.33 | NA |  |
|  | Absence | 44 | 19 | Ref |  |  |  |
|  |  |  |  |  | NA: Not assesed |  |

|  |  |  |  |
| --- | --- | --- | --- |
| **Table 4 Active treatment modality and tumor site** |  |  |  |
|  | **Tumor site** | p value |
|  | **EX** | **AW** | **OT** | **RP** | **Neck** |
| **Treatment modality** |  |  |  |  | 0.05 |
|  MTX + VBL (n = 46) | 14 | 8 | 12 | 2 | 10 |  |
|  Pazopanib (n = 3) | 1 | 0 | 1 | 0 | 1 |  |
|  Surgery (n = 24) | 5 | 14 | 5 | 0 | 0 |  |
|  Oral MTX (n =1) | 1 | 0 | 0 | 0 | 0 |  |

MTX; Methotrexate, VBL; Vinblastine, EX; Extremity, AW; Abdominal wall,

OT; Other trunk, RP; Retroperitoneal.

**Table 5 Studies about active surveillance in desmoid-type fibromatosis**

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Reference | N | Tumor site | PD | Shift to AT | Risk factor |
| Fiore, 200921  | 83 | Extremity 27Trunk 5Head and neck 3Abdominal wall 33Thoracic wall 9Intra-abdominal 6 | 29(35%) |  23(28%) | NS |
| Bonvalot, 201320  | 102 | Abdominal wall 102 | NS | 37(36%) | Size > 3.5cm |
| Colombo, 201522  | 70 | Intra-abdominal 10Exttremity/girdle 26Head/neck 2Trunk 32 | 28(40%) | 28(40%) | NS |
| Burtenshaw, 201627  | 120 | Abdominal wall 63Intra-abdominal 49Both 8 (all FAP patients) | NS | 53 (44%) | NS |
| Penel, 201723  | 388 | NS | 117(30%) | 71(18%) | NS |
| Van Broekhoven, 201825  | 37 | Head/neck 3Thorax/back 13Abdominal wall 13Extremity 4 | 5(14%) | 15 (41%) | NS |
| Turner, 201928  | 50 | Head/neck 3Upper extremity 4Lower Extremity 14Abdominal wall 14Intra-abdominal 3Chest wall 6Breast 2Limb girdle 2Other 2 | 21(42%) | 19(38%) | NS |

**Table 5 (Continued)**

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Reference | N | Tumor site | PD | Shift to AT | Risk factor |
| Van Houdt, 201926  | 168 | Lower extremity 20Upper extremity 31Abdominal wall 61Intra-abdominal 15Chest wall 30Other 11 | 60(36%) | 78(46%) | Size > 7cmPainRECIST PD |
| Cassidy, 202024  | 72 | Abdominal wall 19Chest wall 5Intra-abdominal 21Extremity 21Other 6 | NS | 42(58%) | Size > 5cmParaspinal/frank location |
| Cuomo, 202130  | 70 | Upper extremity 20Lower extremity 22Pelvic gridle 26Shoulder gridle 31 | 38(54%) | NS | PainShoulder location |
| Soboczuk, 202129  | 139 | Abdominal wall 46Chest wall 40Upper extremity 10Lower extremity 25Intra-abdominal 15  | 60(43%) | 41(29%) | NS |
| This study | 168 | Neck 20Extremity 50Abdominal wall 45Other trunk 47Retroperitoneal 6 | 71/156(46%) | 74/168(44%) | Neck location*CTNNB1* S45FRECIST PD |
| AT; active treatment, PD; progressive disease, FAP; Familial Adenomatous Polyposis, NS; not specified |